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Public summary of opinion on orphan designation

Copanlisib for the treatment of marginal zone lymphoma

On 24 August 2018, orphan designation (EU/3/18/2064) was granted by the European Commission to Bayer AG, Germany, for copanlisib 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 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 approximately 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 available 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.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with marginal zone lymphoma because early studies in patients indicated a

*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 517,400,000 (Eurostat 2018).

beneficial effect in patients whose disease had come back after or did not respond to previous 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?

Copanlisib is expected to work by blocking the effects of an enzyme called PI3K which plays a role in the growth and survival of white blood cells. PI3K is overactive in marginal zone lymphoma cells, where it enables the survival of the cancer cells. By targeting this enzyme and blocking its effects, copanlisib is expected to cause death of the cancer cells, thereby delaying or stopping the progression of the cancer.

What is the stage of development of this medicine?

The effects of copanlisib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with copanlisib in patients with marginal zone lymphoma were ongoing.

At the time of submission, copanlisib was authorised in the United States for follicular lymphoma.

At the time of submission, copanlisib was not authorised anywhere in the EU for marginal zone lymphoma. Orphan designation of copanlisib had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 19 July 2018 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's [rare disease designations page](#).

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Copanlisib	Treatment of marginal zone lymphoma
Bulgarian	Копанлисиб	Лечение на маргинално зонален лимфом
Croatian	Kopanlizib	Liječenje limfoma marginalne zone
Czech	Copanlisib	Léčba lymfomu z marginální zóny
Danish	Copanlisib	Behandling af marginalzonelymfom
Dutch	Copanlisib	Behandeling van marginale zone lymfoom
Estonian	Kopanlisiib	Marginaaltsooni lümfoomi ravi
Finnish	Kopanlisiibi	Marginaalivyöhykkeen lymfooman hoito
French	Copanlisib	Traitement du lymphome de la zone marginale
German	Copanlisib	Behandlung des Marginalzonelymphoms
Greek	Κοπανλίσιμπη	Θεραπεία του λεμφώματος μεθοριακής ζώνης
Hungarian	Copanlisib	Marginális zóna lymphoma kezelése
Italian	Copanlisib	Trattamento del linfoma della zona marginale
Latvian	Kopanlisibs	Marginālo zonu limfomas ārstēšana
Lithuanian	Kopanlisibas	Marginalinės zonos limfomos gydymas
Maltese	Kopanlisib	Kura ta' limfoma taż-żona marġinali
Polish	Kopanlizyb	Leczenie chłoniaka strefy brzeżnej
Portuguese	Copanlisib	Tratamento do linfoma da zona marginal
Romanian	Copanlisib	Tratamentul limfomului de zonă marginală
Slovak	Kopanlisib	Liečba lymfómu z marginálnej zóny
Slovenian	Kopanlisib	Zdravljenje limfoma marginalne cone
Spanish	Copanlisib	Tratamiento del linfoma de la zona marginal
Swedish	Kopanlisib	Behandling av marginalzonelymfom
Norwegian	Kopanlisib	Behandling av marginalsonelymfom
Icelandic	Copanlisib	Meðferð við jaðarsvæðiseitlkrabbameini

¹ At the time of designation