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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Idelalisib for the treatment of splenic marginal zone lymphoma

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Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in October 2013 on request of the Sponsor.

On 5 August 2013, orphan designation (EU/3/13/1187) was granted by the European Commission to Gilead Sciences International Ltd, United Kingdom, for idelalisib for the treatment of splenic marginal zone lymphoma.

What is splenic marginal zone lymphoma?

Splenic marginal zone lymphoma is a cancer of a type of white blood cell called B lymphocytes or B cells. In splenic marginal zone lymphoma, the B cells multiply too quickly and live for too long, so there are too many of them. The abnormal B cells affect the spleen, causing it to become enlarged, and the bone marrow (the spongy tissue inside the large bones in the body, where blood cells are produced).

Splenic marginal zone lymphoma is a life-threatening disease and long-term debilitating due to damage to the spleen and bone marrow and the risk of the disease transforming into a more aggressive form of lymphoma.

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

At the time of designation, splenic marginal zone lymphoma affected approximately 0.05 in 10,000 people in the European Union (EU). This was equivalent to a total of around 2,500 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 splenic 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 (anticancer medicines), and radiotherapy (treatment with radiation).

The sponsor has provided sufficient information to show that idelalisib might be of significant benefit for patients with splenic marginal zone lymphoma because early studies show that it might improve the outcome of patients with marginal zone lymphoma whose disease was resistant to or had come back after existing treatments. 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?

Idelalisib blocks the effects of an enzyme called PI3K-delta which is a member of a family of enzymes called phosphoinositide-3-kinases (PI3K) that plays an important role in the growth, migration and survival of white blood cells. PI3K-delta is active in the abnormal B cells of patients with splenic marginal zone lymphoma, stimulating their growth and survival. By blocking its effects, the medicine is expected to reduce the growth and survival of the abnormal B cells.

What is the stage of development of this medicine?

The effects of idelalisib have been evaluated in experimental models.

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

At the time of submission, idelalisib was not authorised anywhere in the EU for splenic marginal zone lymphoma. Orphan designation of idelalisib had been granted in the EU for lymphoplasmacytic lymphoma and follicular lymphoma, and in the United States for chronic lymphocytic leukaemia.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 11 July 2013 recommending the granting of this designation.

[*]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).

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	Idelalisib	Treatment of splenic marginal zone lymphoma
Bulgarian	Иделалисиб	Лечение на спленален маргинално зонален лимфом
Croatian	Idelalizib	Liječenje spleničnog limfoma marginalne zone
Czech	Idelalisib	Léčba splenického lymfomu z marginální zóny
Danish	Idelalisib	Behandling af splenisk marginalzonelymfom
Dutch	Idelalisib	Behandeling van marginale zone lymfoom in de milt
Estonian	Idelalisiib	Põrna marginaaltsooni lümfoomi ravi
Finnish	Idelalisibi	Pernan marginaalivyöhykkeen lymfooman hoito
French	Idelalisib	Traitement des lymphomes spléniques de la zone marginale
German	Idelalisib	Behandlung des splenischen Marginalzonenlymphoms
Greek	Ιδελαλισίμπη	Θεραπεία του σπληνικού λεμφώματος οριακής ζώνης
Hungarian	Idelaliszib	Splenicus marginális zóna lymphoma kezelése
Italian	Idelalisib	Trattamento del linfoma splenico della zona marginale
Latvian	Idelalizibs	Liesas marginālās zonas limfomas ārstēšana
Lithuanian	Idelalisibas	Blužnies marginalinės zonos limfomos gydymas
Maltese	Idelalisib	Kura ta' limfoma taż-żona marġinali splenika
Polish	Idelalizyb	Leczenie pierwotnego śledzionowego chłoniaka strefy brzeżnej
Portuguese	Idelalisib	Tratamento do linfoma da zona marginal esplénica
Romanian	Idelalisib	Tratamentul limfomului de zonă marginală splenică
Slovak	Idelalizib	Liečba lymfómu marginálnej zóny sleziny
Slovenian	Idelalizib	Zdravljenje limfoma marginalne cone vranice
Spanish	Idelalisib	Tratamiento del linfoma esplénico de la zona marginal
Swedish	Idelalisib	Behandling av marginalzonslymfom i mjälten
Norwegian	Idelalisib	Behandling av splenisk marginalsonelymfom
Icelandic	Idelalísíb	Meðferð við jaðarsvæðiseitilkrabbameini (marginal zone lymphoma) í milta

[1] At the time of designation