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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Idelalisib for the treatment of extranodal marginal zone lymphoma of mucosa-associated lymphoid tissue (MALT 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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in October 2013 on request of the Sponsor.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/3/13/1186) was granted by the European Commission to Gilead Sciences International Ltd, United Kingdom, for idelalisib for the treatment of extranodal marginal zone lymphoma of mucosa-associated lymphoid tissue (MALT lymphoma).

What is extranodal marginal zone lymphoma of mucosa-associated lymphoid tissue (MALT lymphoma)?

Extranodal marginal zone lymphoma of mucosa-associated lymphoid tissue (MALT lymphoma) is a cancer of a type of white blood cell called B lymphocytes or B cells. The abnormal B cells multiply too quickly and live for too long, so there are too many of them. In MALT lymphoma the abnormal B cells affect various organs, particularly the gut, but also thyroid gland, lung, breast, eye and skin. Patients usually have fever, weight loss, tiredness and night sweats.

MALT lymphoma is a life-threatening and long-term debilitating disease due to gut problems and the risks of bone marrow damage and of the disease transforming to 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, MALT lymphoma affected approximately 0.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 20,000 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 MALT 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), radiotherapy (treatment with radiation) and surgery to remove affected lymph nodes. In some patients, MALT 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 idelalisib might be of significant benefit for patients with MALT lymphoma because early studies in patients with marginal zone lymphoma show that it might improve the outcome of patients 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. In patients with MALT lymphoma, PI3K-delta is active in the abnormal B cells, 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 MALT lymphoma were ongoing.

At the time of submission, idelalisib was not authorised anywhere in the EU for MALT 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 extranodal marginal zone lymphoma of mucosa-associated lymphoid tissue (MALT lymphoma)
Bulgarian	Иделалисиб	Лечение на екстранодален маргинално зонален лимфом на мукозно-асоциирана лимфоидна тъкан (МАЛТ лимфом)
Croatian	Idelalizib	Liječenje ekstranodalnog limfoma marginalne zone s podrijetlom u limfatičnom tkivu sluznice (MALT limfom)
Czech	Idelalisib	Léčba extranodálního lymfomu z marginální zóny slizniční lymfoidní tkáně (MALT lymfom)
Danish	Idelalisib	Behandling af extranodalt marginalzonelymfom i mucosa-associeret lymfoidt væv (MALT-lymfom)
Dutch	Idelalisib	Behandeling van extranodale marginale zone lymfoom van mucosa-geassocieerd lymfoïd weefsel (MALT-lymfoom)
Estonian	Idelalisiib	Limaskestaga seotud lümfoidse koe ekstranodaalse marginaaltsooni lümfoomi ravi (MALT-lümfoom)
Finnish	Idelalisibi	Ekstranodaalisen marginaalivyöhykkeen lymfooman hoito limakalvoon liittyvässä imukudoksessa (MALT-lymfooma)
French	Idelalisib	Traitement des lymphomes extraganglionnaires de la zone marginale (Lymphomes du tissu lymphoïde associé aux muqueuses – lymphomes MALT)
German	Idelalisib	Behandlung des extranodalen Marginalzonenlymphoms des mukosaassoziierten lymphatischen Gewebes (MALT-Lymphom)
Greek	Ιδελαλισίμπη	Θεραπεία του εξωλεμφαδενικού λεμφώματος της οριακής ζώνης του λεμφικού ιστού των βλεννογόνων (λέμφωμα MALT)
Hungarian	Idelaliszib	A mucosa-asszociált lymphoid szövet extranodalis marginális zóna lymphomájának (MALT-lymphoma) kezelése
Italian	Idelalisib	Trattamento del linfoma della zona marginale extranodale del tessuto linfoide associato alla mucosa (linfoma MALT)
Latvian	Idelalizibs	Ar gļotādām saistīto limfoīdo audu marginālās zonas limfomas ar ekstranodulāru lokalizāciju (MALT limfomas) ārstēšana
Lithuanian	Idelalisibas	Ekstranodulinės marginalinės zonos su gleivine susijusio limfoidinio audinio limfomos gydymas
Maltese	Idelalisib	Kura tal-limfoma taż-żona marġinali extranodali ta' tessut limfojde assoċjat mal-mukuża (MALT lymphoma)
Polish	Idelalizyb	Leczenie pozawęzłowego chłoniaka strefy brzeżnej systemu MALT
Portuguese	Idelalisib	Tratamento de linfoma da zona marginal extranodal do linfoma MALT
Romanian	Idelalisib	Tratamentul limfomului de zonă marginală extranodal al ţesutului limfoid asociat mucoaselor (limfom MALT)
Slovak	Idelalizib	Liečba extranodálneho lymfómu marginálnej zóny slizničného lymfoidného tkaniva (MALT)

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Idelalizib	Zdravljenje ekstranodalnega limfoma marginalne cone, ki izvira iz limfoidnega tkiva sluznice (limfom MALT)
Spanish	Idelalisib	Tratamiento del linfoma extranodal de la zona marginal del tejido linfoide asociado a mucosas (linfoma MALT)
Swedish	Idelalisib	Behandling av extranodalt marginalzonslymfom i mucosaassocierad lymfoid vävnad (MALT-lymfom)
Norwegian	Idelalisib	Behandling av ekstranodalt marginalsonelymfom i mucosaassosiert lymfoid vev (MALT-lymfom)
Icelandic	Idelalísíb	Meðferð við jaðarsvæðiseitilkrabbameini (marginal zone lymphoma) utan eitla, í slímhúðar-tengdum eitilvef (MALT lymfóma)