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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Idelalisib for the treatment of follicular 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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in October 2013 on request of the Sponsor.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 17 July 2013, orphan designation (EU/3/13/1159) was granted by the European Commission to Gilead Sciences International Ltd, United Kingdom, for idelalisib 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 and long-term debilitating disease due to organ damage and the cancer coming back.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7418 8416
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

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.

The sponsor has provided sufficient information to show that idelalisib might be of significant benefit for patients with follicular lymphoma, because early studies in patients with follicular lymphoma that was resistant to or had come back after existing treatments showed that some of these patients responded to idelalisib. 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 play 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 follicular 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 follicular lymphoma were ongoing.

At the time of submission, idelalisib was not authorised anywhere in the EU for follicular lymphoma or designated as an orphan medicinal product elsewhere for this condition. Orphan designation of idelalisib had been granted 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 13 June 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:

Gilead Sciences International Limited
Flowers Building
Granta Park
Abington
Cambridge CB21 6GT
United Kingdom
Telephone: +44 122 3897 300
Telefax: +44 122 3897 284
E-mail: regulatory.orphan@gilead.com

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 follicular lymphoma
Bulgarian	Иделалисиб	Лечение на фоликуларен лимфом
Czech	Idelalisib	Léčba folikulárního lymfomu
Croatian	Idelalizib	Liječenje folikularnog limfoma
Danish	Idelalisib	Behandling af follikulært lymfom
Dutch	Idelalisib	Behandeling van folliculair lymfoom
Estonian	Idelalisiib	Follikulaarse lümfoomi ravi
Finnish	Idelalisibi	Follikulaarisen lymfooman hoito
French	Idelalisib	Traitement des lymphomes folliculaires
German	Idelalisib	Behandlung des follikulären Lymphoms
Greek	Ιδελαλισίμπη	θεραπεία του θυλακιώδους λεμφώματος
Hungarian	Idelalis-zib	Follicularis lymphoma kezelése
Italian	Idelalisib	Trattamento del linfoma follicolare
Latvian	Idelalizibs	Folikulārās limfomas ārstēšana
Lithuanian	Idelalisibas	Folikulinės limfomos gydymas
Maltese	Idelalisib	Kura tal-limfoma follikulari
Polish	Idelalizyb	Leczenie chłoniaków grudkowych
Portuguese	Idelalisib	Tratamento do linfoma folicular
Romanian	Idelalisib	Tratamentul limfomului folicular
Slovak	Idelalisib	Liečba folikulárneho lymfómu
Slovenian	Idelalizib	Zdravljenje folikularnega limfoma
Spanish	Idelalisib	Tratamiento del linfoma folicular
Swedish	Idelalisib	Behandling av follikulärt lymfom
Norwegian	Idelalisib	Behandling av follikulært lymfom
Icelandic	Idelalísíb	Meðferð á follicular eitilfrumukrabbameini

[1] At the time of designation