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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Idelalisib for the treatment of chronic lymphocytic leukaemia / small lymphocytic 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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in October 2013 on request of the Sponsor.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/3/13/1173) was granted by the European Commission to Gilead Sciences International Ltd, United Kingdom, for idelalisib for the treatment of chronic lymphocytic leukaemia / small lymphocytic lymphoma.

What is chronic lymphocytic leukaemia / small lymphocytic lymphoma?

Chronic lymphocytic leukaemia (CLL) is cancer of a type of white blood cell called B lymphocytes. In this disease, the lymphocytes multiply too quickly and live for too long, so that there are too many of them circulating in the blood. The cancerous lymphocytes look normal, but they are not fully developed and do not work properly. Over a period of time, the abnormal cells replace the normal white cells, red cells and platelets (components that help the blood to clot) in the bone marrow (the spongy tissue inside the large bones in the body, where blood cells are produced). The disease known as 'small lymphocytic lymphoma' (SLL) is essentially the same disease as CLL. The name SLL is normally used when the cancer cells are located mainly in the lymph nodes.

CLL / SLL is the most common type of leukaemia and mainly affects older people. It is rare in people under the age of 40 years.

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CLL / SLL is a long-term debilitating and life-threatening disease because it involves the weakening of the immune system (the body's natural defences), which can leave patients vulnerable to severe infections.

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

At the time of designation, CLL / SLL affected less than 3.5 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 178,000 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?

Treatment for CLL / SLL is complex and depends on a number of factors, including the extent of the disease, whether it has been treated before, and the patient's age, symptoms and general state of health. Patients whose CLL / SLL is not causing any symptoms or is only getting worse very slowly may not need treatment. Treatment for CLL / SLL is only started if symptoms become troublesome. At the time of designation, the main treatment for CLL / SLL was chemotherapy (medicines to treat cancer).

The sponsor has provided sufficient information to show that idelalisib might be of significant benefit for patients with CLL / SLL because early studies show that it might improve the outcome of patients whose disease does not respond to or has come back after 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?

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

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 CLL / SLL were ongoing.

At the time of submission, idelalisib was not authorised anywhere in the EU for CLL / SLL. Orphan designation of idelalisib had been granted in the United States for CLL and in the EU for lymphoplasmacytic lymphoma and follicular lymphoma.

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 chronic lymphocytic leukaemia / small lymphocytic lymphoma
Bulgarian	Иделалисиб	Лечение на хронична лимфоцитна левкемия/дребноклетъчен лимфоцитен лимфом
Croatian	Idelalizib	Liječenje kronične limfocitne leukemije/limfoma malih stanica
Czech	Idelalisib	Léčba chronické lymfocytické leukémie / lymfomu z malých lymfocytů
Danish	Idelalisib	Behandling af kronisk lymfocytær leukæmi/småcellet lymfocytært lymfom
Dutch	Idelalisib	Behandeling van chronische lymfocytaire leukemie/klein lymfocytair lymfoom
Estonian	Idelalisiib	Kroonilise lümfotsütaarse leukeemia / väikserakulise lümfotsütaarse lümfoomi ravi
Finnish	Idelalisibi	Kroonisen lymfosyyttisen leukemian/pienilymfosyyttisen lymfooman hoito
French	Idelalisib	Traitement de la leucémie lymphoïde chronique/du lymphome lymphocytaire à petites cellules
German	Idelalisib	Behandlung der chronischen lymphatischen Leukämie / des kleinzelligen lymphozytischen Lymphoms
Greek	Ιδελαλισίμπη	Θεραπεία χρόνιας λεμφοκυτταρικής λευχαιμίας/ λεμφώματος από μικρά λεμφοκύτταρα
Hungarian	Idelalisszib	Krónikus lymphocytás leukaemia/kis lymphocytás lymphoma kezelése
Italian	Idelalisib	Trattamento della leucemia linfatica cronica/del linfoma a piccoli linfociti
Latvian	Idelalizibs	Hroniskas limfocitārās leikēmijas/mazo šūnu limfocitārās limfomas ārstēšana
Lithuanian	Idelalisibas	Lėtinės limfocitinės leukemijos / smulkių limfocitų limfomos gydymas
Maltese	Idelalisib	Kura tal-lewkimja limfoċitika kronika/limfoma limfoċitika żgħira
Polish	Idelalizyb	Leczenie przewlekłej białaczki limfatycznej/chłoniaków limfocytarnych z małych limfocytów
Portuguese	Idelalisib	Tratamento da leucemia linfocítica crónica/ linfoma linfocítico de pequenas células
Romanian	Idelalisib	Tratamentul leucemiei limfocitare cronice/limfomului limfocitar cu celule mici
Slovak	Idelalizib	Liečba chronickej lymfocytovej leukémie / lymfómu z malých lymfocytov
Slovenian	Idelalizib	Zdravljenje kronične limfocitne levkemije/drobnoceličnega limfocitnega limfoma

[1] At the time of designation

Language	Active ingredient	Indication
Spanish	Idelalisib	Tratamiento de la leucemia linfocítica crónica/del linfoma linfocítico pequeño
Swedish	Idelalisib	Behandling av kronisk lymfocytisk leukemi /småcelligt lymfocytiskt lymfom
Norwegian	Idelalisib	Behandling av kronisk lymfatisk leukemi / småcellet lymfocytært lymfom
Icelandic	Idelalísíb	Meðferð við langvinnu eitilfrumuhvítblæði/smáeitilfrumukrabbameini