21 January 2014
EMA/COMP/708125/2013
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Ibrutinib for the treatment of follicular lymphoma

On 16 January 2014, orphan designation (EU/3/13/1212) was granted by the European Commission to Janssen-Cilag International N.V., Belgium, for ibrutinib 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. It can also transform into a more aggressive form of the cancer.

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

At the time of designation, follicular lymphoma affected less than 3.6 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 184,000 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.

*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 512,200,000 (Eurostat 2013).

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

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with follicular lymphoma because early studies in patients show that it might improve the outcome of patients whose disease has come back or is no longer responding to treatment with other medicines. 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?

Ibrutinib is expected to work in patients with follicular lymphoma by blocking the action of an enzyme known as Bruton's tyrosine kinase (BTK). BTK is important for the growth and survival of B cells, including the abnormal B cells of the cancer, and their migration to the organs where these cells normally divide. By blocking the action of BTK, it is expected that the medicine will slow the migration of abnormal B cells and induce cell death, thereby slowing the progression of the disease.

What is the stage of development of this medicine?

The effects of ibrutinib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with ibrutinib in patients with follicular lymphoma were ongoing.

At the time of submission, ibrutinib was not authorised anywhere in the EU for follicular lymphoma or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 6 November 2013 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:

Janssen-Cilag International N.V.
Turnhoutseweg, 30
2340 Beerse
Belgium
Tel. +32 146 031 89
Fax +32 146 055 33
E-mail: http://www.janssen-emea.com/contactus

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	Ibrutinib	Treatment of follicular lymphoma
Bulgarian	Ибрутиниб	Лечение на фоликуларен лимфом
Czech	Ibrutinib	Léčba folikulárního lymfomu
Croatian	Ibrutinib	Liječenje folikularnog limfoma
Danish	Ibrutinib	Behandling af follikulært lymfom
Dutch	Ibrutinib	Behandeling van folliculair lymfoom
Estonian	Ibrutiniib	Follikulaarse lümfoomi ravi
Finnish	Ibrutinibi	Follikulaarisen lymfooman hoito
French	Ibrutinib	Traitement des lymphomes folliculaires
German	Ibrutinib	Behandlung des follikulären Lymphoms
Greek	Ιβρουτινίμπη	θεραπεία του θηλακιώδους λεμφώματος
Hungarian	Ibrutinib	Follicularis lymphoma kezelése
Italian	Ibrutinib	Trattamento del linfoma follicolare
Latvian	Ibrutinibs	Folikulārās limfomas ārstēšana
Lithuanian	Ibrutinibas	Folikulinės limfomos gydymas
Maltese	Ibrutinib	Kura tal-limfoma follikulari
Polish	Ibrutynib	Leczenie chłoniaków grudkowych
Portuguese	Ibrutinib	Tratamento do linfoma folicular
Romanian	Ibrutinib	Tratamentul limfomului folicular
Slovak	Ibrutinib	Liečba folikulárneho lymfómu
Slovenian	Ibrutinib	Zdravljenje folikularnega limfoma
Spanish	Ibrutinib	Tratamiento del linfoma folicular
Swedish	Ibrutinib	Behandling av follikulärt lymfom
Norwegian	Ibrutinib	Behandling av follikulært lymfom
Icelandic	Íbrútíníb	Meðferð á follicular eitilfrumukrabbameini

[1] At the time of designation