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EMA/COMP/3/2003 Rev.4
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Iodine (^{131}I) tositumomab for the treatment of follicular lymphoma

First publication	25 May 2013
Rev.1: administrative update	26 May 2003
Rev.2: transfer of sponsorship	12 December 2005
Rev.3: sponsor's change of address	17 June 2011
Rev.4: withdrawal from the Community Register	17 April 2015

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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in March 2015 on request of the Sponsor.

On 14 February 2003, orphan designation (EU/3/03/136) was granted by the European Commission to Amersham plc, U.K., for iodine (^{131}I) tositumomab for the treatment of follicular lymphoma.

The sponsorship was transferred to GlaxoSmithKline Research & Development Limited, United Kingdom, in August 2005.

What is follicular lymphoma?

Lymphoma is a disease in which malignant (cancer) cells form in the lymphatic system. The lymphatic system consists of the tissues and organs that produce, store, and carry white blood cells. These cells fight infection and other diseases. There are several different types of lymphomas. In follicular lymphoma, the cancer cells are related to white blood cells called B-lymphocytes. Follicular lymphoma is a life-threatening disease.

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

At the time of designation, follicular lymphoma affected approximately 3.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 138,000 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 submission of the application for orphan drug designation, several medicinal products were authorised in the European Union for use in follicular lymphoma. The choice of treatment depends in particular on the extension of the disease as well as on the responses to therapies previously prescribed. Iodine (^{131}I) tositumomab might be of potential significant benefit for the treatment of follicular lymphoma in association with tositumomab, particularly in terms of efficacy in advanced stages. The assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

Iodine (^{131}I) tositumomab is an antibody which can carry a radioactive particle (iodine-131). Antibodies are proteins which specifically recognise and attach themselves to certain foreign substances, such as proteins found on the surface of cancer cells or bacteria. In the case of tositumomab, the antibody targets a specific structure on B-cell lymphocytes involved in follicular lymphoma called the CD20 receptor. The antibody is expected to bind to the cancer cells and to kill them with the radiation from the iodine.

What is the stage of development of this medicine?

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

Iodine (^{131}I) tositumomab in association with tositumomab was granted orphan drug status in the United States of America on 16 May 1994 for the indication of treatment of B-cell non-Hodgkin's lymphoma. Iodine (^{131}I) tositumomab was not marketed anywhere worldwide for follicular lymphoma, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 10 January 2003 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.
At the time of designation, this represented a population of 382,800,000 (Eurostat 2003).

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	Iodine (^{131}I) tositumomab	Treatment of follicular lymphoma
Czech	Tositumomab, značený jodem (^{131}I)	Léčba folikulárního lymfomu
Danish	Jod (^{131}I) tositumomab	Behandling af follikulært lymfom
Dutch	Iodine (^{131}I) tositumomab	Behandeling van folliculair lymfoom
Estonian	Jood (^{131}) tositumomab	Follikulaarse lümfoomi ravi
Finnish	Jodi (^{131}I) tositumomabi	Follikulaarisen lymfooman hoito.
French	Tositumomab marqué à l'iode (^{131}I)	Traitement des lymphomes folliculaires
German	Iod (^{131}I) Tositumomab	Behandlung von follikulärem Lymphom
Greek	Ιώδιο (^{131}I) tositumomab	Θεραπεία οζώδους λεμφώματος
Hungarian	^{131}I-tositumomab	Folliculáris lymphoma kezelése
Italian	Iodio (^{131}I) tositumomab	Trattamento del linfoma follicolare
Latvian	Jodīna (^{131}I) tositumomabs	Folikulārās limfomas ārstēšana
Lithuanian	Jodo (^{131}I) tositumomabas	Folikulinės limfomos gydymas
Polish	Jodowany (^{131}I) tositumomab	Leczenie chłoniaka grudkowego
Portuguese	Iodo (^{131}I) tositumomab	Tratamento de linfoma folicular
Slovak	Jód (^{131}I) tositumomab	Liečba folikulárneho lymfómu
Slovenian	Jod (^{131}I) tositumomab	Zdravljenje folikularnega limfoma
Spanish	Yodo (^{131}I) tositumomab	Tratamiento del linfoma folicular
Swedish	Jod (^{131}I) tositumomab	Behandling av follikulärt lymfom

[1] At the time of transfer of sponsorship