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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Blinatumomab for the treatment of acute lymphoblastic leukaemia

First publication	8 September 2009
Rev.1: sponsor's name change	21 February 2013
Rev.2: transfer of sponsorship	30 April 2014

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.

On 24 July 2009, orphan designation (EU/3/09/650) was granted by the European Commission to Micromet AG, Germany, for blinatumomab for the treatment of acute lymphoblastic leukaemia.

In January 2012, Micromet AG changed name to Micromet GmbH. In May 2012, Micromet GmbH changed name to AMGEN Research (Munich) Gmbh.

The sponsorship was transferred to Amgen Europe BV, The Netherlands, in February 2014.

What is acute lymphoblastic leukaemia?

Acute lymphoblastic leukaemia (ALL) is a cancer of the white blood cells called lymphocytes. In this disease, the lymphocytes multiply too quickly and live for too long, so there are too many of them circulating in the blood. These abnormal lymphocytes are not fully developed and do not work properly. Over a period of time, they replace the normal white cells and red blood cells and platelets in the bone marrow (the spongy tissue inside the large bones in the body).

ALL is the most common type of leukaemia in young children, but the disease also affects adults, especially those aged 65 years and older. Many people with ALL can be cured. However, despite the available treatments, ALL remains a serious and life-threatening disease in some patients.

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

At the time of designation, acute lymphoblastic leukaemia affected approximately 1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 50,000 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 ALL 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. At the time of designation, the main treatment of ALL was chemotherapy (medicines used to kill cancer cells) followed by or combined with radiotherapy (using radiations to kill cancer cells). Bone marrow transplantation was also used. This is a complex procedure where the bone marrow of the patient is destroyed and replaced with healthy bone marrow from a matched donor.

The sponsor has provided sufficient information to show that blinatumomab might be of significant benefit for patients with ALL because it works in a different way to existing treatments and preliminary studies indicate that it may be an alternative to 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?

Blinatumomab is a monoclonal antibody (a type of protein) that has been designed to specifically recognise and attach to the following proteins:

- CD19, a protein that is found on the surface of ALL cells;
- the 'T cell receptor/CD3 complex', which is responsible for the activation of some cells of the immune system (the body's natural defences) called T cells.

By attaching to the cancer cells and the T cell receptor/CD3 complex, the medicine is expected to stimulate the T cells to kill the cancer cells.

What is the stage of development of this medicine?

The effects of blinatumomab have been evaluated in experimental models.

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

At the time of submission, blinatumomab was not authorised anywhere in the EU for ALL. Orphan designation of blinatumomab had been granted in the United States of America for ALL.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 2 June 2009 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.
At the time of designation, this represented a population of 504,800,000 (Eurostat 2009).

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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Fax: 31 76 5 732001
http://www.amgen.nl/dutch/contact_us/amgen_contact.jsp

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	Blinatumomab	Treatment of acute lymphoblastic leukaemia
Bulgarian	Блинатумомаб	Лечение на остра лимфобластна левкемия
Croatian	Blinatumomab	Liječenje akutne limfoblastične leukemije
Czech	Blinatumomab	Léčba akutní lymfoblastické leukémie
Danish	Blinatumomab	Behandling af akut lymfoblastær leukæmi
Dutch	Blinatumomab	Behandeling van acute lymfoblastaire leukemie
Estonian	Blinatumomaab	Ägeda lümfoblastilise leukeemia ravi
Finnish	Blinatumomabi	Akuutin lymfoblastileukemian hoito
French	Blinatumomab	Traitement de la leucémie lymphoblastique aiguë
German	Blinatumomab	Behandlung der akuten lymphatischen Leukämie
Greek	Μπλινατουμομάμπη	Θεραπεία της οξείας λεμφοβλαστικής λευχαιμίας
Hungarian	Blinatumomab	Akut lymphoblastos leukaemia kezelése
Italian	Blinatumomab	Trattamento della leucemia linfoblastica acuta
Latvian	Blinatumomabs	Akūtas limfoblastiskas leikozes ārstēšana
Lithuanian	Blinatumomabas	Ūmios limfoblastinės leukemijos gydymas
Maltese	Blinatumomab	Kura tal-lewkimja limfoblastika akuta
Polish	Blinatumomab	Leczenie ostrej białaczki limfoblastycznej
Portuguese	Blinatumomab	Tratamento da leucémia linfoblástica aguda
Romanian	Blinatumomab	Tratamentul leucemiei limfoblastice acute
Slovak	Blinatumomab	Liečba akútnej lymfoblastickej leukémie
Slovenian	Blinatumomab	Zdravljenje akutne limfoblastne levkemije
Spanish	Blinatumomab	Tratamiento de la leucemia linfoblástica aguda
Swedish	Blinatumomab	Behandling av akut lymfatisk leukemi
Norwegian	Blinatumomab	Behandling av akutt lymfoblastisk leukemi
Icelandic	Blínatúmómab	Meðferð við bráðu eitilfrumuhvítblæði

[1] At the time of transfer of sponsorship