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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Lintuzumab for the treatment of acute myeloid leukaemia

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in August 2011 on request of the Sponsor.

On 30 April 2009, orphan designation (EU/3/09/627) was granted by the European Commission to Seattle Genetics UK Limited, United Kingdom, for lintuzumab for the treatment of acute myeloid leukaemia.

What is acute myeloid leukaemia?

Acute myeloid leukaemia (AML) is a cancer of the white blood cells (the cells that fight infections). In patients with AML, the bone marrow (the spongy tissue inside the large bones) produces large numbers of abnormal, immature white blood cells called 'blasts'. These abnormal cells quickly build up in large numbers in the bone marrow and are found in the blood.

AML is a life-threatening disease because these immature cells take the place of the normal white blood cells, reducing the patient's ability to fight infections.

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

At the time of designation, AML affected less than 2 in 10,000 people in the European Union (EU)*. This is equivalent to a total of fewer than 101,000 people, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

Treatment for AML 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

*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 504,800,000 (Eurostat 2009).

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the time of designation, the main treatments for AML were chemotherapy (medicines to kill cancer cells) and bone marrow transplantation (a complex procedure where the bone marrow of the patient is destroyed and replaced with bone marrow from a matched donor).

The sponsor has provided sufficient information to show that lintuzumab might be of significant benefit for patients with AML because it works in a different way to existing 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?

Lintuzumab is a monoclonal antibody. A monoclonal antibody is an antibody (a type of protein) that has been designed to recognise and attach to a specific structure (called an antigen) that is found on certain cells in the body. Lintuzumab has been designed to attach to CD33, a receptor that is found on the surface of most of the blast cells in AML. By attaching to the receptor, lintuzumab is expected to activate certain cells in the immune system (the body's natural defences), so that they kill the blasts. This is expected to slow down the development of AML.

What is the stage of development of this medicine?

The effects of lintuzumab have been evaluated in experimental models.

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

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 4 March 2009 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:

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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	Lintuzumab	Treatment of acute myeloid leukaemia
Bulgarian	Линтузумаб	Лечение на остра миелоидна левкемия
Czech	Lintuzumab	Léčba akutní myeloidní leukémie
Danish	Lintuzumab	Behandling af akut myeloid leukæmi
Dutch	Lintuzumab	Behandeling van acute myeloïde leukemie
Estonian	Lintusumaab	Akuutse müeloidse leukeemia ravi
Finnish	Lintutsumabi	Akuutin myelooisen leukemian hoito
French	Lintuzumab	Traitement de la leucémie aiguë myéloïde
German	Lintuzumab	Behandlung der akuten myeloischen Leukämie
Greek	Lintuzumab	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	Lintuzumab	Akut myeloid leukaemia kezelése
Italian	Lintuzumab	Trattamento della leucemia mieloide acuta
Latvian	Lintuzumabs	Akūtas mieloleikozes ārstēšana
Lithuanian	Lintuzumabas	Ūmios mieloleukozės gydymas
Maltese	Lintuzumab	Kura tal-lewkimja mjelojda akuta
Polish	Lintuzumab	Leczenie ostrej białaczki szpikowej
Portuguese	Lintuzumab	Tratamento da leucémia mielóide aguda
Romanian	Lintuzumab	Tratamentul leucemiei mieloide acute
Slovak	Lintuzumab	Liečba akútnej myeloickej leukémie
Slovenian	Lintuzumab	Zdravljenje akutne mieloične levkemije
Spanish	Lintuzumab	Tratamiento de la leucemia mieloide aguda
Swedish	Lintuzumab	Behandling av akut myeloisk leukemi
Norwegian	Lintuzumab	Behandling av akutt myelogen leukemi
Icelandic	Lintuzúmab	Meðferð við bráðu kyrningahvítblæði

[1] At the time of designation