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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Lintuzumab for the treatment of myelodysplastic syndromes

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in August 2011 on request of the Sponsor.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 29 April 2009, orphan designation (EU/3/09/626) was granted by the European Commission to Seattle Genetics UK Limited, United Kingdom, for lintuzumab for the treatment of myelodysplastic syndromes.

What are myelodysplastic syndromes?

Myelodysplastic syndromes (MDSs) are a group of disorders in which the red blood cells, white blood cells and platelets produced by the bone marrow (the spongy tissue inside the large bone) do not grow and mature normally. Patients with MDSs can develop several symptoms including tiredness or weakness due to anaemia (low red blood cell counts), infections due to low white blood cells, and bruising or abnormal bleeding due to low platelet counts.

MDSs are life-threatening diseases because they can lead to severe anaemia, infections or bleeding, and can result in leukaemia (cancer of the white blood cells).

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

At the time of designation, MDSs affected approximately 2.3 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 116,000 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).

*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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What treatments are available?

At the time of designation, some medicines were authorised in the EU for the treatment of MDSs. The choice of treatment for MDSs depends on a number of factors including the type and the extent of the disease, whether it has been treated before, and the patient's age, symptoms and general state of health. Current treatments for MDSs include bone marrow transplants and chemotherapy (medicines used to kill cancer cells).

The sponsor has provided sufficient information to show that lintuzumab might be of significant benefit for patients with MDS 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 bind 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 abnormal cells in the blood and bone marrow. 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 abnormal blood cells. This is expected to slow down the development of MDSs.

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 MDSs were ongoing.

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

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 myelodysplastic syndromes
Bulgarian	Линтузумаб	Лечение на миелодиспластичен синдром
Czech	Lintuzumab	Léčba myelodysplastického syndromu
Danish	Lintuzumab	Behandling af myelodysplastiske syndromer
Dutch	Lintuzumab	Behandeling van myelodysplastische syndromen
Estonian	Lintusumaab	Müelodüsplastiliste sündroomide ravi
Finnish	Lintutsumabi	Myelodysplastisten syndroomien hoito
French	Lintuzumab	Traitement des syndromes myélodysplasiques
German	Lintuzumab	Behandlung der myelodysplastischen Syndrome
Greek	Lintuzumab	Θεραπεία των μυελοδυσπλαστικών συνδρόμων
Hungarian	Lintuzumab	Myelodysplasias syndroma kezelése
Italian	Lintuzumab	Trattamento delle sindromi mielodisplastiche
Latvian	Lintuzumabs	Mielodisplastisko sindromu ārstēšana
Lithuanian	Lintuzumabas	Mielodisplastinių sindromų gydymas
Maltese	Lintuzumab	Kura tas-sindromi mjelodisplastiċi
Polish	Lintuzumab	Leczenie zespołów mielodysplastycznych
Portuguese	Lintuzumab	Tratamento dos síndromes mielodisplásicos
Romanian	Lintuzumab	Tratamentul sindromului mielodisplazic
Slovak	Lintuzumab	Liečba myelodysplastického syndrómu
Slovenian	Lintuzumab	Zdravljenje mielodisplastičnega sindroma
Spanish	Lintuzumab	Tratamiento de los síndromes mielodisplásicos
Swedish	Lintuzumab	Behandling av myelodysplastiska syndrom
Norwegian	Lintuzumab	Behandling av myelodysplastisk syndrom
Icelandic	Lintuzúmab	Til meðferðar við mergmisþroskaheilkenni

[1] At the time of designation