



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Moxetumomab pasudotox for the treatment of B-lymphoblastic leukaemia/lymphoma

On 17 July 2013, orphan designation (EU/3/13/1150) was granted by the European Commission to MedImmune Ltd, United Kingdom, for moxetumomab pasudotox for the treatment of B-lymphoblastic leukaemia/lymphoma.

What is B-lymphoblastic leukaemia/lymphoma?

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

B-lymphoblastic leukaemia/lymphoma is a long-term debilitating and life-threatening disease because the abnormal immature cells take the place of the normal white blood cells, reducing the patient's ability to fight infections and causing organ damage.

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

At the time of designation, B-lymphoblastic leukaemia/lymphoma affected less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 51,000 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 B-lymphoblastic leukaemia/lymphoma 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, treatment for B-lymphoblastic

*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 509,000,000 (Eurostat 2013).



leukaemia/lymphoma included chemotherapy (medicines to treat cancer) that may be followed by or combined with radiotherapy (treatment with radiation). Haematopoietic (blood) stem-cell transplantation was also used. This is a complex procedure where the patient receives stem cells from a matched donor to help restore the bone marrow.

The sponsor has provided sufficient information to show that moxetumomab pasudotox might be of significant benefit for patients with B-lymphoblastic leukaemia/lymphoma because it selectively targets the abnormal B cells and early studies indicate that it might benefit patients who are not responding to previous 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?

Moxetumomab pasudotox is made up of two components:

- a shortened form of pseudomonas exotoxin, a substance toxic for cells (produced by the bacterium *Pseudomonas aeruginosa*);
- a monoclonal antibody (a type of protein) that has been designed to recognise and attach to a protein on the surface of B cells that regulates their survival and interaction with other cells of the immune system.

Once attached to the B cell, the medicine is expected to be taken up by the cell, where the exotoxin becomes active, causing breaks in the cell DNA and thereby killing the cell.

What is the stage of development of this medicine?

The effects of moxetumomab pasudotox have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with moxetumomab pasudotox in patients with B-lymphoblastic leukaemia/lymphoma were ongoing.

At the time of submission, moxetumomab pasudotox was not authorised anywhere in the EU for B-lymphoblastic leukaemia/lymphoma. Orphan designation of moxetumomab pasudotox has been granted in the EU for treatment of hairy cell leukaemia, and in the United States for acute lymphoblastic leukaemia, hairy cell leukaemia and for chronic lymphocytic leukemia.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 June 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:

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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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Moxetumomab pasudotox	Treatment of B-lymphoblastic leukaemia/lymphoma
Bulgarian	Моксетумомаб пасудотокс	Лечение на В-лимфобластна левкемия/лимфом
Czech	Moxetumomab pasudotox	Léčba B-lymfoblastické leukémie/lymfomu
Croatian	Moksetumomab pasudotoks	Liječenje B-limfoblastične leukemije/limfoma
Danish	Moxetumomab pasudotox	Behandling af B-lymfoblastisk leukæmi/lymfom
Dutch	Moxetumomab pasudotox	Behandeling van B-lymfoblastische leukemie/lymfoom
Estonian	Moxetumomab pasudotox	B-rakulise lümfoblastilise leukeemia/lümfoomi ravi
Finnish	Moksetumomabipasudotoksi	B-lymfoblastileukemia/-lymfooman hoito
French	Moxetumomab pasudotox	Traitement de la leucémie/du lymphome lymphoblastique de type B
German	Moxetumomab pasudotox	Behandlung der B-lymphoblastischen Leukämie/des B-lymphoblastischen Lymphoms
Greek	Μοξετουμομάμπη πασουντοτόξη	Θεραπεία Β-λεμφοβλαστικής λευχαιμίας/λεμφώματος
Hungarian	Moxetumomab paszudotox	B-sejtes lymphoblastos leukaemia/lymphoma kezelése
Italian	Moxetumomab pasudotox	Trattamento della leucemia linfoblastica B/linfoma linfoblastico B
Latvian	Moksetumomaba pasudotoks	B šūnu limfoleikozes/limfomas ārstēšana
Lithuanian	Moksetumomabo pasudotoksas	B limfoblastinės leukemijos / limfomos gydymas
Maltese	Moxetumomab pasudotox	Kura ta' lewkimja/limfoma limfoblastika-B
Polish	Moksetumomab pasudotoks	Leczenie białaczki/chłoniaka limfoblastycznego z limfoblastów B
Portuguese	Moxetumomab pasudotox	Tratamento da leucémia/linfoma linfoblástico B
Romanian	Moxetumomab pasudotox	Tratamentul leucemiei/limfomului cu celule limfoblastice tip B
Slovak	Moxetumomab pasudotox	Liečba B-lymfoblastickej leukémie/lymfómu
Slovenian	Moksetumomab pasudotoks	Zdravljenje B-limfoblastne levkemije/limfoma
Spanish	Moxetumomab pasudotox	Tratamiento de la leucemia / linfoma linfoblástico de células B
Swedish	Moxetumomab pasudotox	Behandling av B-cellsleukemi/B-cellslymfom

¹ At the time of designation

Language	Active ingredient	Indication
Norwegian	Moksetumomabpasudotoks	Behandling av B-lymfoblastisk leukemi/lymfom
Icelandic	Moxetúmómab pasúdótox	Meðferð við B-eitilfrumuhvítblæði/eitilfrumukrabbameini

Withdrawn