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Public summary of opinion on orphan designation

Lutetium (^{177}Lu) lilotomab satetraxetan for the treatment of marginal zone lymphoma

On 4 June 2020, orphan designation EU/3/20/2280 was granted by the European Commission to Nordic Nanovector ASA, Norway, for lutetium (^{177}Lu) lilotomab satetraxetan (also known as Betalutin) for the treatment of marginal zone lymphoma.

What is marginal zone lymphoma?

Marginal zone lymphoma is a cancer of a type of white blood cell called B lymphocytes or B cells. In marginal zone lymphoma, abnormal B cells multiply quickly and live for too long. The abnormal B cells affect various organs. Patients usually have fever, weight loss, tiredness and night sweats.

Marginal zone lymphoma is a life-threatening and long-term debilitating disease due to its effects on the spleen, lymph nodes and bone marrow, as well as the increased risk of infection.

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

At the time of designation, marginal zone lymphoma affected approximately 1.7 in 10,000 people in the European Union (EU). This was equivalent to a total of around 88,000 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 designation, the main treatments for marginal zone lymphoma available in the EU included chemotherapy (cancer medicines), immunotherapy (using the body's own immune system to kill cancer cells) with the medicine rituximab, radiotherapy (treatment with radiation) and surgery.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with marginal zone lymphoma. Early data show that patients whose cancer has come back or failed to respond to several other treatments responded well to lutetium (^{177}Lu) lilotomab

*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, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).

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satetraxetan. 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?

This medicine contains an antibody (a type of protein) called lilotomab and the radioactive substance lutetium, which releases small amounts of radiation. When the medicine is given to the patient, the antibody attaches to a protein called CD37 found on the surface of cancer cells, while lutetium releases radiation which is expected to kill the cancer cells.

What is the stage of development of this medicine?

The effects of lutetium (^{177}Lu) lilotomab satetraxetan have been evaluated in experimental models.

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

At the time of submission, the medicine was not authorised anywhere in the EU for the treatment of marginal zone lymphoma or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 23 April 2020, 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website.

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	Lutetium (^{177}Lu) lilotomab satetraxetan	Treatment of marginal zone lymphoma
Bulgarian	Лутециев (^{177}Лу) лилотомаб сатетраксетан	Лечение на маргинално зонален лимфом
Croatian	Lutecij (^{177}Lu) lilotomab satetraksetan	Liječenje limfoma marginalne zone
Czech	Lutetium (^{177}Lu) lilotomab satetraxetan	Léčba lymfomu z marginální zóny
Danish	Lutetium (^{177}Lu) lilotomab satetraxetan	Behandling af marginalzonelymfom
Dutch	Lutetium (^{177}Lu) lilotomab satetraxetan	Behandeling van marginale zone lymfoom
Estonian	Luteetsium (^{177}Lu) lilotomab satetraxetaan	Marginaaltsooni lümfoomi ravi
Finnish	Lutetium (^{177}Lu) lilotomabi-satetraksetaani	Marginaalivyöhykkeen lymfooman hoito
French	Lutetium (^{177}Lu) lilotomab satetraxetan	Traitement du lymphome de la zone marginale
German	Lutetium (^{177}Lu) lilotomab satetraxetan	Behandlung des Marginalzonenlymphoms
Greek	Λουτέτσιο (^{177}Lu) λιλοτομάμπησατετραξετάνη	Θεραπεία του λεμφώματος μεθοριακής ζώνης
Hungarian	Lutécium (^{177}Lu) lilotomab satetraxetan	Marginális zóna lymphoma kezelése
Italian	Lutezio (^{177}Lu) lilotomab satetraxetan	Trattamento del linfoma della zona marginale
Latvian	Lutēcija (^{177}Lu) lilotomaba satetraksetāns	Marginālo zonu limfomas ārstēšana
Lithuanian	Liutecio (^{177}Lu) lilotomabas satetraksetanas	Marginalinės zonos limfomos gydymas
Maltese	Lutezju (^{177}Lu) lilotomab satetraksetan	Kura ta' limfoma taż-żona marġinali
Polish	Lutetu (^{177}Lu) lilotomab satetraksetan	Leczenie chłoniaka strefy brzeżnej
Portuguese	Lutécio (^{177}Lu) lilotomab satetraxetan	Tratamento do linfoma da zona marginal
Romanian	Lutetium (^{177}Lu) lilotomab satetraxetan	Tratamentul limfomului de zonă marginală
Slovak	Lutécium (^{177}Lu) lilotomab satetraxetan	Liečba lymfómu z marginálnej zóny
Slovenian	Lutetium (^{177}Lu) lilotomab satetraksetan	Zdravljenje limfoma marginalne cone
Spanish	Lutetium (^{177}Lu) lilotomab satetraxetan	Tratamiento del linfoma de la zona marginal
Swedish	Lutetium (^{177}Lu) lilotomab satetraxetan	Behandling av marginalzonslymfom
Norwegian	Lutetium (^{177}Lu) lilotomab satetraksetan	Behandling av marginalsonelymfom
Icelandic	Lútetíumuríum (^{177}Lu) lilotomabsatetraxetan	Meðferð við jaðarsvæðiseitilkrabbameini

[1] At the time of designation