



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Lutetium (^{177}Lu) edotreotide for the treatment of gastro-entero-pancreatic neuroendocrine tumours

On 4 June 2014, orphan designation (EU/03/14/1269) was granted by the European Commission to ITG Isotope Technologies Garching GmbH, Germany, for lutetium (^{177}Lu) edotreotide for the treatment of gastro-entero-pancreatic neuroendocrine tumours.

What are gastro-entero-pancreatic neuroendocrine tumours?

Gastro-entero-pancreatic neuroendocrine tumours (GEP-NETs) are tumours that arise from neuroendocrine cells in the gut. These cells release hormones that control various functions of the digestive system. The symptoms of GEP-NETs depend on where the tumour is located within the gut and on whether it produces excess hormones. Often by the time of diagnosis the tumours have spread to other organs such as the liver.

GEP-NETs are debilitating as they often produce excess hormones that may cause severe symptoms. They are life-threatening if they spread to other organs in the body.

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

At the time of designation, GEP-NETs affected approximately 3.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 179,000 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 the application for orphan designation, several products were authorised in the EU for treating GEP-NETs, including octreotide, everolimus, and sunitinib.

*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 28), Norway, Iceland and Liechtenstein. This represents a population of 511,100,000 (Eurostat 2014).



The sponsor has provided sufficient information to show that lutetium (¹⁷⁷Lu) edotreotide might be of significant benefit for patients with GEP-NETs because data showed that patients treated with the medicine lived for longer without their disease getting worse. 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?

Most tumour cells of patients with GEP-NETs have high amounts of receptors for a hormone called somatostatin. Lutetium (¹⁷⁷Lu) edotreotide has two main components, one of which, edotreotide, has a similar structure to somatostatin and can attach to the same receptors on the tumour cells, while the other is a radioactive form of the substance lutetium (¹⁷⁷Lu). By attaching to the tumour cells and then by delivering a small amount of short-range radiation from the lutetium, the medicine is expected to be able to kill the tumour cells, and thereby stop or slow down the progression of the disease.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of lutetium (¹⁷⁷Lu) edotreotide in experimental models was ongoing.

At the time of submission, no clinical trials with the medicine in patients with GEP-NETs had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for GEP-NETs or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 April 2014 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	Lutetium (¹⁷⁷ Lu) edotreotide	Treatment of gastro-entero-pancreatic neuroendocrine tumours
Bulgarian	Лутеций (¹⁷⁷ Lu) едотреотид	Лечение на гастро-ентеро-панкреатични невроендокринни тумори
Croatian	Lutecijev(¹⁷⁷ Lu) edotreotid	Liječenje gastroenteropankreatičnih neuroendokrinih tumora
Czech	Lutetium (¹⁷⁷ Lu) edotreotide	Léčba gastro-entero- pankreatických neuroendokrinních tumorů
Danish	Lutetium (¹⁷⁷ Lu) edotreotid	Behandling af gastro-entero-pancreatiske neuroendocrine tumorer
Dutch	Lutetium (¹⁷⁷ Lu) edotreotide	Behandeling van gastro-entero-pancreatische neuroendocriene tumoren
Estonian	Luteetsium (¹⁷⁷ Lu) edotreotiid	Gastroenteropankreaatiliste neuroendokriintuumorite ravi
Finnish	Lutetium(¹⁷⁷ Lu)edotreotidi	Maha-suolikanavan ja haiman neuroendokriinisten kasvainten hoito
French	Lutetium (¹⁷⁷ Lu) edotrétotide	Traitement des tumeurs neuroendocrines gastroentéropancréatiques
German	Lutetium (¹⁷⁷ Lu) Edotreotide	Behandlung von gastro-entero-pankreatischen neuroendokrinen Tumoren
Greek	Λουτέσιο (¹⁷⁷ Lu) εδοτρεοτιδη	Θεραπεία των γαστρεντεροπαγκρεατικών νευροενδοκρινικών όγκων
Hungarian	¹⁷⁷ Lu-tetraxetan-edotreotid	Gasztro-entero-pankreatikus neuroendokrin tumorok kezelése
Italian	Lutetium (¹⁷⁷ Lu) edotreotide	Trattamento dei tumori gastro-entero-pancreatici neuroendocrini
Latvian	Lutetiuma (¹⁷⁷ Lu) edotreotīds	Kuņģa-zarnu -aizkuņģa dziedzerā neiroendokrīnu audzēju ārstēšana
Lithuanian	Lutecio (¹⁷⁷ Lu) edotreotidas	Skrandžio-žarnyno-kasos neuroendokrininių navikų gydymas
Maltese	Lutetium (¹⁷⁷ Lu) edotreotide	Kura ta' tumuri newroendokrini gastro-entero-pankrejatiċi
Polish	Lutetetu (¹⁷⁷ Lu) edotreotyd	Leczenie guzów neuroendokrynnych przewodu pokarmowego i trzustki
Portuguese	Lutécio (¹⁷⁷ Lu) edotreotide	Tratamento dos tumores neuroendócrinos gastro-entero-pancreáticos
Romanian	Lutetium (¹⁷⁷ Lu) edotreotidă	Tratamentul tumorilor neuroendocrine gastro-entero-pancreatice
Slovak	Lutécium (¹⁷⁷ Lu) edotreotid	Liečba gastroenteropankreatických neuroendokrinných nádorov

¹ At the time of designation

Language	Active ingredient	Indication
Slovenian	Lutetium (¹⁷⁷ Lu) edotreotid	Zdravljenje gastro-entero-pancreatičnih neuroendokrinih tumorjev
Spanish	Edotreotida marcado con Lutecio (¹⁷⁷ Lu)	Tratamiento de los tumores neuroendocrinos gastroenteropancreáticos
Swedish	Lutetium (¹⁷⁷ Lu) edotreotid	Behandling av gastroenteropankreatiska neuroendokrina tumörer
Norwegian	Lutetium (¹⁷⁷ Lu) edotreotid	Behandling av gastroenteropankreatiske nevroendokrine tumorer
Icelandic	Lútetíum (¹⁷⁷ Lú) edótreótíð	Meðferð á maga-þarma- bris æxlum af taugainnkirtla-toga