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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Yttrium (^{90}Y) edotreotide for the treatment of gastro-entero-pancreatic neuroendocrine tumours

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Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

On 4 December 2008, orphan designation (EU/3/08/589) was granted by the European Commission to Molecular Insight Pharmaceuticals GmbH, Germany, for yttrium (^{90}Y) edotreotide for the treatment of gastro-entero-pancreatic neuroendocrine tumours.

The sponsorship was transferred to Molecular Insight Limited, United Kingdom, in June 2010 and subsequently to ITG Isotope Technologies Garching GmbH, Germany, in July 2013.

What are gastro-entero-pancreatic neuroendocrine tumours?

Gastro-entero-pancreatic neuroendocrine tumours (GEP-NETs) are tumours of the 'neuroendocrine system' in the gut. This is the system where the nervous system and hormonal system interact to control the digestive organs. These tumours share a number of common characteristics, for example content of specific chemical substances called "neuroendocrine markers". There are two main types of GEP-NETs: carcinoid tumours, and "pancreatic-type" endocrine tumours (these often occur in the pancreas, but also in other sites). GEP-NETs are debilitating as they often secrete 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, gastro-entero-pancreatic neuroendocrine tumours affected approximately 1.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 80,000



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 the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of submission of the application for orphan designation, several medicines were authorised in the EU for the treatment of one or more types of GEP-NET. In most cases, these medicines act on the symptoms caused by the secretion of hormones, but are not active against the growth of the tumours. Commonly, surgery to remove the tumours and treatment with 'somatostatin analogues' were used. Somatostatin analogues are similar to a naturally occurring hormone somatostatin that prevents the release of many hormones.

The sponsor has provided sufficient information to show that yttrium (^{90}Y) edotreotide might be of significant benefit for patients because it is expected to kill tumour cells, reduce tumour size and eventually improve the long term outcome of the patients. This assumption will have to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Yttrium (^{90}Y) edotreotide contains a somatostatin analogue called edotreotide that has been tagged with a radioactive form of the chemical element yttrium called yttrium-90 (^{90}Y). The somatostatin analogue is expected to attach to the tumour cells, where the radiation from the ^{90}Y is expected to act locally to kill the tumour cells.

What is the stage of development of this medicine?

The effects of yttrium (^{90}Y) edotreotide have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with gastro-entero-pancreatic neuroendocrine tumours were ongoing.

At the time of submission, yttrium (^{90}Y) edotreotide was not authorised anywhere in the world for gastro-entero-pancreatic neuroendocrine tumours. Orphan designation of yttrium (^{90}Y) edotreotide had been granted in the United States of America for the treatment of somatostatin-positive gastro-entero-pancreatic neuroendocrine tumours.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 October 2008 recommending the granting of this designation.

*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.
At the time of designation, this represented a population of 502,800,000 (Eurostat 2008).

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 European Union) 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:

ITG Isotope Technologies Garching GmbH
Lichtenbergstr. 1
85748 Garching
Germany
Tel: +49 89 289 13921
Fax: +49 89 289 13929

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	Yttrium (90Y) edotreotide	Treatment of gastro-entero-pancreatic neuroendocrine tumours
Bulgarian	Итрий (90Y) едотреотид	Лечение на гастро-ентеро-панкреатични невроендокринни тумори
Croatian	Itrijev (⁹⁰ Y) edotreotid	Za liječenje gastroenteropankreatičnih neuroendokrinih tumora
Czech	Edotreotid yttria (90Y)	Léčba gastroenteropankreatických neuroendokrinních tumorů
Danish	Yttrium (90Y) edotreotid	Behandling af gastroentero pankreatiske neuroendokrine tumorer
Dutch	Yttrium (90Y) edotreotide	Behandeling van gastro-entero-pancreatische neuro-endocriene tumoren
Estonian	Üttrium (90Y) edotreotiid	Gastroenteropankreataatiliste neuroendokriintuumorite ravi
Finnish	Yttrium (90Y) -edotreotidi	Maha-suolikanavan ja haiman neuroendokriinisten kasvainten hoito
French	Édotréotide marqué à l'yttrium (90Y)	Traitement des tumeurs neuro-endocrines gastro-entéro-pancréatiques
German	Yttrium (90Y) Edotreotid	Behandlung von gastro-entero-pankreatischen neuroendokrinen Tumoren
Greek	Εδοτρεοτιδη ιττρίου (90Y)	Θεραπεία των γαστρεντεροπαγκρεατικών νευροενδοκρινικών όγκων
Hungarian	Yttrium (90Y) edotreotid	Gastro-entero-pancreaticus neuroendokrin tumorok kezelése
Italian	Ittrio (90Y) edotreotide	Trattamento dei tumori neuroendocrini gastroenteropancratici
Latvian	Itrija (90Y) edotreotīds	Kuņģa-zarnu trakta-aizkuņģa dziedzerā neiroendokrīnu audzēju ārstēšana
Lithuanian	Itrio (90Y) edotreotidas	Skrandžio, žarnų, kasos neuroendokrininių navikų gydymas
Maltese	Yttrium (90Y) edotreotide	Kura ta' tumuri newroendokrini gastro-entero-pankrejatiċi
Polish	Edotreotyd itru (90Y)	Leczenie pacjentów z guzami neuroendokrynnymi przewodu pokarmowego i trzustki
Portuguese	Itrio (90Y) edotreotida	Tratamento de tumores neuroendócrinos gastro-entero-pancreáticos
Romanian	Ytriu (90Y) edotreotid	Tratamentul tumorilor neuroendocrine gastro-entero-pancreatice
Slovak	Yttrium (90Y) edotreotid	Liečba gastroenteropankreatických neuroendokrinných tumorov

¹ At the time of transfer of sponsorship

Language	Active Ingredient	Indication
Slovenian	Edotreotid itrija (90Y)	Zdravljenje gastroenteropankreatičnih neuroendokrinih tumorjev
Spanish	Itrio (90Y) edotreotida	Tratamiento de los tumores neuroendocrinos gastroenteropancreáticos
Swedish	Yttrium (90Y) edotreotid	Behandling av neuroendokrina tumörer i mage, tarm och bukspottkörtel
Norwegian	Yttrium (90Y) edotreotid	Behandling av gastro-entero-pankreatiske neuroendokrine tumorer
Icelandic	Ýttríum (90Y) edótreotíð	Til meðferðar við maga-þarma- bris æxlum af taugainnkirtla-toga