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EMA/COMP/125762/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Gallium (^{68}Ga)-edotreotide for the diagnosis of gastro-entero-pancreatic neuroendocrine tumours

On 19 March 2015, orphan designation (EU/3/15/1450) was granted by the European Commission to Advanced Accelerator Applications SA, France, for gallium (^{68}Ga)-edotreotide for the diagnosis 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 long-term 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 eligible for diagnosis?

At the time of designation, the number of patients expected to use the medicine for diagnosis of GEP-NETs was estimated to be approximately 3.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 180,000 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 methods of diagnosis are available?

At the time of designation, GEP-NETs were diagnosed using various methods. These included histopathology (examining a tissue under the microscope) and biochemical testing (measuring substances produced by neuroendocrine tumours), as well as imaging methods such as magnetic

*Disclaimer: For the purpose of the designation, the number of patients elegible for diagnosis of 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 512,900,000 (Eurostat 2015).

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resonance imaging (MRI) and computer tomography (CT) to visualise the location of the tumour. Somatostatin receptor scintigraphy was a commonly used imaging technique, employing a radioactive tracer to obtain an image. At the time of designation, ^{111}In-DTPA-pentetreotide (Octreoscan) was authorised in the EU for use in scintigraphy.

The sponsor has provided sufficient information to show that the medicinal product might be of significant benefit for patients with GEP-NETs because early studies in experimental models indicate that it may improve the accuracy of detecting tumours compared with somatostatin receptor scintigraphy. 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 is to be used for an imaging method called positron emission tomography (PET). It consists of a radioactive element, gallium (^{68}Ga), attached to a substance containing a somatostatin analogue, a substance similar to a natural hormone called somatostatin. Most GEP-NETs have high amounts of somatostatin receptors on their surface. The product is expected to attach to these somatostatin receptors and to accumulate in the GEP-NETs cells. These cells are then expected to emit radiation that can be detected by the PET imaging method. This allow to determine the location of the tumour and if it has spread to other parts of the body.

What is the stage of development of this medicine?

The effects of the product have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the product in patients with GEP-NETs had been started.

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 February 2015 recommending the granting of this designation.

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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	Gallium (^{68}Ga)-edotreotide	Diagnosis of gastro-entero-pancreatic neuroendocrine tumours
Bulgarian	Галий (^{68}Га) едотреотид	Диагностика на гастро-ентеро-панкреатични невроендокринни тумори
Croatian	Galijev[^{68}Ga] edotreotid	Dijagnoza gastroenteropankreatičnih neuroendokrinih tumora
Czech	Gallium (^{68}Ga) edotreotide	Diagnosa gastro-entero- pankreatických neuroendokrinních tumorů
Danish	Gallium (^{68}Ga)-edotreotid	Diagnose af gastro-entero-pancreatiske neuroendocrine tumorer
Dutch	Gallium (^{68}Ga)-edotreotide	Diagnose van gastro-entero-pancreatische neuroendocriene tumoren
Estonian	Gallium (^{68}Ga)-edotreotiid	Gastroenteropankreaatiliste neuroendokriintuumorite diagnoosimine
Finnish	Gallium (^{68}Ga)-edotreotidi	Maha-suolikanavan ja haiman neuroendokriinisten kasvainten diagnosointi
French	Gallium (^{68}Ga) edotréotide	Diagnostic des tumeurs neuro-endocrines gastro-entéro-pancréatiques
German	Gallium (^{68}Ga)-Edotreotid	Diagnose von gastro-entero-pankreatischen neuroendokrinen Tumoren
Greek	Γάλλιο (^{68}Ga)-εδοτρεοτίδη	Διάγνωση των γαστρεντεροπαγκρεατικών νευροενδοκρινικών όγκων
Hungarian	Gallium (^{68}Ga)-edotreotid	Gasztro-entero-pankreatikus neuroendokrin tumorok diagnosztizálása
Italian	Gallio (^{68}Ga)-edotreotide	Diagnosi di tumori gastro-entero-pancreatici neuroendocrini
Latvian	Gallija (^{68}Ga)-edotreotīds	Kuņģa-zarnu trakta-aizkuņģa dziedzera neiroendokrīnu audzēju diagnostikai
Lithuanian	Galio (^{68}Ga) edotreotidas	Skrandžio-žarnyno-kasos neuroendokrininių tumorų ligos nustatymas
Maltese	Gallium (^{68}Ga)-edotreotide	Dijanjosi ta' tumuri newroendokrini gastro-entero-pankrejatiċi
Polish	Gal (^{68}Ga)-edotreotyd	Diagnostyka guzów neuroendokrynnych przewodu pokarmowego
Portuguese	Gálio (^{68}Ga)-edotreotida	Diagnóstico dos tumores neuroendócrinos gastro-entero-pancreáticos
Romanian	Galiu (^{68}Ga)-edotreotid	Diagnosticul tumorilor neuroendocrine gastro-entero-pancreatice
Slovak	Gálium (^{68}Ga) edotreotid	Diagnostika gastroenteropankreatických neuroendokrinných nádorov

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Galijev (^{68}Ga)-edotreotid	Diagnosticiranje gastro-entero-pancreatičnih neuroendokrinih tumorjev
Spanish	Galio (^{68}Ga)-Edotreotida	Diagnostico de los tumores neuroendocrinos gastroenteropancreáticos
Swedish	Gallium (^{68}Ga) edotreotid	Diagnostik av gastroenteropankreatiska neuroendokrina tumörer
Norwegian	Gallium (^{68}Ga)-edotreotid	Diagnostikk av gastroenteropankreatiske nevroendokrine tumorer
Icelandic	Gallíum (^{68}Ga)-edótreótíð	Greining á maga-þarma-bris æxlum af taugainnkirtla¬toga