



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

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

On 27 October 2011, orphan designation (EU/3/11/920) was granted by the European Commission to OctreoPharm Sciences GmbH, Germany, for gallium (^{68}Ga)-pasireotide tetraxetan 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 of the 'neuroendocrine system' in the gut. This is where the nervous and hormonal systems interact to control the digestive organs. GEP-NETs share a number of common characteristics, for example they share 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 may also be found 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 gastro-entero-pancreatic neuroendocrine tumours?

At the time of designation, the number of patients eligible for diagnosis of gastro-entero-pancreatic neuroendocrine tumours was estimated to be less than 3.7 in 10,000 people in the European Union (EU)*. This is equivalent to a total of fewer than 187,000 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. They included histopathology (examining a tissue specimen under the microscope) and biochemical testing (using chemical markers to measure substances produced by neuroendocrine tumours). Sometimes imaging

*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 506,300,000 (Eurostat 2011).



methods such as magnetic resonance imaging (MRI) and computer tomography (CT) were used 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, the only agent authorised for use in scintigraphy was ^{111}In -DTPA-pentetreotide (Octreoscan).

The sponsor has provided sufficient information to show that Gallium (^{68}Ga)-pasireotide tetraxetan might be of significant benefit for patients with GEP-NETs because early studies indicate that when used as an agent for scintigraphy it may improve the accuracy of diagnosis compared with existing methods. 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?

The product is an agent to be used for an imaging method called Positron Emission Tomography (PET). The product consists of a radioactive element, gallium (^{68}Ga), attached to pasireotide tetraxetan, a man-made substance which is similar to a natural hormone called somatostatin. Many somatostatin receptors are found on the surface of GEP-NETs cells. Once injected into a vein, gallium (^{68}Ga)-pasireotide tetraxetan is expected to attach to these receptors on the GEP-NETs cells, and to emit radiation that can be detected by the PET imaging method, thereby allowing the tumour to be diagnosed.

What is the stage of development of this medicine?

The effects of gallium (^{68}Ga)-pasireotide tetraxetan were evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with gallium (^{68}Ga)-pasireotide tetraxetan in patients with GEP NETs had been started.

At the time of submission, gallium (^{68}Ga)-pasireotide tetraxetan 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 8 September 2011 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 | Gallium (⁶⁸ Ga)-pasireotide tetraxetan | Diagnosis of gastro-entero-pancreatic neuroendocrine tumours |
| Bulgarian | Галий (⁶⁸ Га)-пасиреотид тетраксетан | Диагностициране на гастро-ентеро-панкреатични невроендокринни тумори |
| Czech | Gallium (⁶⁸ Ga) pasireotid tetraxetan | Diagnostika gastroenteropankreatických neuroendokrinních tumorů |
| Danish | Gallium (⁶⁸ Ga)-pasireotide tetraxetan | Diagnose af gastroentero pankreatiske neuroendokrine tumorer |
| Dutch | Gallium (⁶⁸ Ga)-pasireotide tetraxetan | Diagnose van gastro-entero-pancreatische neuro-endocriene tumoren |
| Estonian | Gallium (⁶⁸ Ga)-pasireotiid tetraksetaan | Gastroenteropankreaatiliste neuroendokriintuumorite diagnoosimine |
| Finnish | Gallium (⁶⁸ Ga)-pasireotiditetraksetaani | Maha-suolikanavan ja haiman neuroendokriinisten kasvainten diagnosointi |
| French | Gallium (⁶⁸ Ga) Pasiréotide tétraxétan | Diagnostic des tumeurs neuro-endocrines gastro-entéro-pancréatiques |
| German | Gallium (⁶⁸ Ga)-Pasireotid-tetraxetan | Diagnose von gastro-entero-pankreatischen neuroendokrinen Tumoren |
| Greek | Γάλλιο (⁶⁸ Γά)-τετραξετανο-πασιρεοτιδῆ | Διάγνωση των γαστρεντεροπαγκρεατικών νευροενδοκρινικών όγκων |
| Hungarian | Gallium (⁶⁸ Ga)-paszireotid-tetraxetán | Gastro-entero-pancreaticus neuroendokrin tumorok diagnosztikája |
| Italian | Gallio (⁶⁸ Ga)-pasireotide tetraxetan | Diagnosi dei tumori neuroendocrini gastroenteropancreatici |
| Latvian | Gallija (⁶⁸ Ga)-pazireotīda tetraksetāns | Kuņģa-zarnu trakta-aizkuņģa dziedzera neiroendokrīnu audzēju diagnostikai |
| Lithuanian | Galio (⁶⁸ Ga) pazireotido tetraksetanas | Skrandžio, žarnų, kasos neuroendokrininių navikų diagnostikai |
| Maltese | Gallium (⁶⁸ Ga)-pasireotide tetraxetan | Dijanżosi ta' tumuri newroendokrini gastro-entero-pankrejatiċi |
| Polish | Gal (⁶⁸ Ga)-pazyreotyd tetraksetan | Diagnostyka guzów neuroendokrynych przewodu pokarmowego i trzustki |
| Portuguese | Gálio (⁶⁸ Ga)-pasireotideo tetraxetano | Diagnóstico de tumores neuroendócrinos gastro-entero-pancreáticos |
| Romanian | Galiu (⁶⁸ Ga)-pasireotid tetraxetan | Diagnosticul tumorilor neuroendocrine gastro-entero-pancreatice |
| Slovak | Gálium (⁶⁸ Ga) pasireotid tetraxetán | Diagnóza gastroentero-pankreatických neuroendokrinných tumorov |
| Slovenian | Galij (⁶⁸ Ga)-pasireotid tetraksetan | Diagnostika gastroenteropankreatičnih neuroendokrinih tumorjev |
| Spanish | Galio (⁶⁸ Ga)-pasireotide tetraxetano | Diagnóstico de los tumores neuroendocrinos gastroenteropancreáticos |

¹ At the time of designation

| Language | Active ingredient | Indication |
|-----------|--|--|
| Swedish | Gallium (⁶⁸ Ga) pasireotidtraxetan | Diagnos av neuroendokrina tumörer i mage, tarm och bukspottkörtel |
| Norwegian | Gallium (68Ga)-pasireotid- tetraksetan | Diagnose av gastroenteropankreatiske neuroendokrine tumorer |
| Icelandic | Gallíum (⁶⁸ Ga)-pasíreotíð tetraxetan | Greining á maga-þarma- bris æxlum af taugainnkirtla-toga |