6 January 2017
EMA/745295/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$ for the diagnosis of gastrointestinal stromal tumours

On 12 December 2016 orphan designation (EU/3/16/1794) was granted by the European Commission to Advanced Accelerator Applications, France, for ^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$ (also known as ^{68}Ga-NeoBOMB1) for the diagnosis of gastrointestinal stromal tumours.

What are gastrointestinal stromal tumours?

Gastrointestinal stromal tumours (GISTs) belong to a group of cancers of the stomach and bowel called sarcomas, which are characterised by uncontrolled growth of cells in the supporting tissues of these organs. GISTs are most common in the stomach (60%), followed by the small intestine (30%), and then the colon and rectum (5%). GISTs occur predominantly in middle-aged and older persons, and are considered a life-threatening condition because the tumours could come back and also spread to other organs.

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 GISTs was approximately 0.15 in 10,000 people in the European Union (EU). This was equivalent to a total of around 8,000 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, no medicines were authorised in the EU for the diagnosis of GIST. Non-specific diagnostic methods such as computer tomography (CT), magnetic resonance imaging (MRI) and endoscopy were used.

*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 513,700,000 (Eurostat 2016).

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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 similar to a 'gastrin releasing peptide'. Most GISTs have high amounts of receptors for gastrin releasing peptide on their surface. By attaching to these receptors, the medicine is expected to build up in the GIST cells and the radioactive substance in the medicine can be detected by PET. This helps to determine the tumours' location and if they have spread to other parts of the body.

What is the stage of development of this medicine?

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

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with GISTs had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for the diagnosis of GISTs 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 4 November 2016 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

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	^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$	Diagnosis of gastrointestinal stromal tumours
Bulgarian	^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$	Диагностика на гастро-ентеро-панкреатични невроендокринни тумори
Croatian	^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$	Dijagnoza gastroenteropankreatičnih neuroendokrinih tumora
Czech	^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$	Diagnosa gastro-entero- pankreatických neuroendokrinních tumorů
Danish	^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$	Diagnose af gastro-entero-pancreatiske neuroendocrine tumorer
Dutch	^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$	Diagnose van gastro-entero-pancreatische neuroendocriene tumoren
Estonian	^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$	Gastroenteropankreaatiliste neuroendokriintuumorite diagnoosimine
Finnish	^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$	Maha-suolikanavan ja haiman neuroendokriinisten kasvainten diagnosointi
French	^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$	Diagnostic des tumeurs neuro-endocrines gastro-entéro-pancréatiques
German	^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$	Diagnose von gastro-entero-pankreatischen neuroendokrinen Tumoren
Greek	^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$	Διάγνωση των γαστρεντεροπαγκρεατικών νευροενδοκρινικών όγκων
Hungarian	^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$	Gasztro-entero-pankreatikus neuroendokrin tumorok diagnosztizálása
Italian	^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$	Diagnosi di tumori gastro-entero-pancreatici neuroendocrini
Latvian	^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$	Kuņģa-zarnu trakta-aizkuņģa dziedzera neiroendokrīnu audzēju diagnostikai
Lithuanian	^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$	Skrandžio-žarnyno-kasos neuroendokrininių tumorų ligos nustatymas
Maltese	^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$	Dijanjosi ta' tumuri newroendokrini gastro-entero-pankrejatiċi
Polish	^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$	Diagnostyka guzów neuroendokrynnych przewodu pokarmowego
Portuguese	^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$	Diagnóstico dos tumores neuroendócrinos gastro-entero-pancreáticos

[1] At the time of designation

Language	Active ingredient	Indication
Romanian	^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$	Diagnosticul tumorilor neuroendocrine gastro-entero-pancreatice
Slovak	^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$	Diagnostika gastroenteropankreatických neuroendokrinných nádorov
Slovenian	^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$	Diagnosticiranje gastro-entero-pancreatičnih neuroendokrinih tumorjev
Spanish	^{68}Ga-DOTA-pABzA-DIG-dFe-Gln-Trp-Ala-Val-Gli-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$	Diagnostico de los tumores neuroendocrinos gastroenteropancreáticos
Swedish	^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$	Diagnostik av gastroenteropankreatiska neuroendokrina tumörer
Norwegian	^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Gly-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$	Diagnostikk av gastrointestinale stromale tumorer
Icelandic	^{68}Ga-DOTA-pABzA-DIG-dPhe-Gln-Trp-Ala-Val-Glý-His-NHCH[(CH_2-$CH(CH_3)_2$]$_2$	Greining á grunnfrumuæxlum í meltingarfærum