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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Florilglutamic acid (^{18}F) for the diagnosis of glioma

On 21 March 2016, orphan designation (EU/3/16/1631) was granted by the European Commission to Piramal Imaging GmbH, Germany, for florilglutamic acid (^{18}F) for the diagnosis of glioma.

What is glioma?

Glioma is a type of brain tumour that affects the 'glial' cells (the cells that surround and support the nerve cells). Patients with glioma can have severe symptoms, but the types of symptoms experienced depend on where the tumour develops in the brain.

Symptoms can include headaches, nausea, vomiting, loss of appetite and changes in personality, mood, mental capacity and concentration. About one fifth of patients with glioma have seizures (fits) for months or years before the disease is diagnosed.

Glioma is a long-term debilitating and life-threatening disease because of the severe damage to the brain, and is associated with poor long-term survival.

What is the estimated number of patients eligible for diagnosis of the condition?

At the time of designation, the number of patients eligible for diagnosis of glioma was estimated to be approximately 1.5 people in 10,000 in the European Union (EU). This was equivalent to a total of around 77,000 people*, and is below the ceiling for orphan designation. 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, several imaging methods were used for the diagnosis of glioma, such as magnetic resonance imaging (MRI), computer tomography (CT) and positron emission tomography (PET) using so-called contrast agents to obtain better images of organs and tissues. Contrast agents

*Disclaimer: For the purpose of the designation, the number of patients eligible 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 513,700,000 (Eurostat 2016).

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approved for the diagnosis of glioma included fludeoxyglucose (^{18}F), 6-[18F]fluoro-L-3,4-dihydroxyphenylalanine (FDOPA) and 5-aminolevulinic acid (Gliolan).

The sponsor has provided sufficient information to show that florilglutamic acid (^{18}F) might be of significant benefit for patients with glioma, with preliminary data showing it could lead to higher image sensitivity than that obtained with other authorised products used in PET scans. It may also be better for detecting tumours when compared with authorised products used in MRI. 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?

Florilglutamic acid (^{18}F) is a radioactive substance for use in PET imaging. Florilglutamic acid (^{18}F) enters glioma cells through a transport system known as x_C- which is more abundant in cancer tissue. When injected into the patient, florilglutamic acid (^{18}F) is more effectively taken up by the glioma cells in the brain from where it is expected to emit radiation that can be detected in a PET scan, thereby allowing the cancer to be diagnosed.

What is the stage of development of this medicine?

The effects of florilglutamic acid (^{18}F) have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicine in patients with glioma were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for hepatocellular carcinoma 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 18 February 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	Florilglutamic acid (^{18}F)	Diagnosis of glioma
Bulgarian	Флорилглутаминова киселина (^{18}F)	Диагностика на глиом
Croatian	Fluoril glutaminska kiselina (^{18}F)	Dijagnosticiranje glioma
Czech	Florilglutamová kyselina (^{18}F)	Diagnóza gliomu
Danish	Florilglutaminsyre (^{18}F)	Diagnose af gliom
Dutch	Florilglutaminezuur (^{18}F)	Diagnose van glioma
Estonian	Fluoriilglutamiinhape (^{18}F)	Glioomi diagnoosimiseks
Finnish	Florilglutamiinihappo[^{18}F]	Gliooman diagnosointi
French	Acide florilglutamique (*^{18}F*)	Diagnostic des gliomes
German	Fluoropropyl-Glutaminsäure (^{18}F)	Diagnose des Glioms
Greek	Φθοριλογλουταμινικό οξύ (^{18}F)	διάγνωση του γλοιώματος
Hungarian	Florilglutaminsav (^{18}F)	Glioma diagnosztizálása
Italian	Acido florilglutamico (^{18}F)	Diagnosi del glioma
Latvian	Florilglutamīnskābe (^{18}F)	Gliomas diagnostika
Lithuanian	Florilglutamo rūgštis (^{18}F)	Gliomos diagnozė
Maltese	Florilglutamic acid (^{18}F)	Dijanjosi tal-glioma
Polish	Kwas florylglutaminowy (^{18}F)	Rozpoznawanie glejaka
Portuguese	Ácido florilglutâmico (^{18}F)	Diagnóstico do glioma
Romanian	Acid fluorilglutamic (^{18}F)	Diagnosticul gliomului
Slovak	Kyselina Florilglutámová (^{18}F)	Diagnóza gliómu
Slovenian	Florilglutamična kislina (^{18}F)	Diagnosticiranje glioma
Spanish	Ácido florilglutámico (^{18}F)	Diagnóstico del glioma
Swedish	Florilglutaminsyra (^{18}F)	Diagnos av gliom
Norwegian	Florilglutaminsyre (^{18}F)	Diagnose av gliom
Icelandic	Flórilglútamínsýra (^{18}F)	Sjúkdómsgreining glióma

[1] At the time of designation