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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Tetrofosmin for the diagnosis of glioma

On 14 October 2016, orphan designation (EU/3/16/1764) was granted by the European Commission to ProActina, Greece, for tetrofosmin 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 depend on where the tumour develops in the brain.

Symptoms can include headaches, nausea (feeling sick), loss of appetite, vomiting, 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 1.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 82,000 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, several imaging methods were used for the diagnosis of glioma, such as magnetic resonance imaging (MRI), computed tomography (CT) and positron emission tomography (PET) using so-called contrast agents or radioactive tracers to obtain better images of organs and

*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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tissues. Agents 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 tetrofosmin might be of significant benefit for patients with glioma, with published data showing it could improve recognition of advanced tumour. 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?

Tetrofosmin is combined with a radioactive substance (technetium 99m) before being used for imaging. The combination is given by injection and normally does not enter the brain as it cannot pass the blood-brain barrier that separates the blood from brain tissue. However, in patients with glioma the proteins that are important for maintaining a tight barrier are no longer functioning properly, leading to gaps in the barrier. This means that tetrofosmin can enter the brain in areas where the cancer is active. It is then taken up by the glial cells and the radioactivity can then be picked up by the scanner. The images obtained are expected to help show the extent of the cancer. The small amount of radioactivity in the medicine disappears quickly and has little effect on the body.

What is the stage of development of this medicine?

The sponsor has provided non-clinical and clinical data from the published literature to support its application for orphan designation.

At the time of submission, tetrofosmin was authorised in the EU for heart and breast tumour imaging.

At the time of submission, tetrofosmin was not authorised anywhere in the EU for diagnosis of glioma 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 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	Tetrofosmin	Diagnosis of glioma
Bulgarian	Тетрофосмин	Диагностика на глиом
Croatian	Tetrofosmin	Dijagnosticiranje glioma
Czech	Tetrofosmin	Diagnóza gliomu
Danish	Tetrofosmin	Diagnose af gliom
Dutch	Tetrofosmine	Diagnose van glioma
Estonian	Tetrofosmiin	Glioomi diagnoosimiseks
Finnish	Tetrofosmini	Gliooman diagnosointi
French	Tétrofosmin	Diagnostic des gliomes
German	Tetrofosmin	Diagnose des Glioms
Greek	Τετροφοσμίνη	Διάγνωση του γλοιώματος
Hungarian	Tetrofosmin	Glioma diagnosztizálása
Italian	Tetrofosmina	Diagnosi del glioma
Latvian	Tetrafosmīns	Gliomas diagnostika
Lithuanian	Tetrofosminas	Gliomos diagnozė
Maltese	Tetrofosmin	Dijanjosi tal-glioma
Polish	Tetrofosmina	Rozpoznawanie glejaka
Portuguese	Tetrofosmina	Diagnóstico do glioma
Romanian	Tetrofosmin	Diagnosticul gliomului
Slovak	Tetrofosmín	Diagnóza gliómu
Slovenian	Tetrofozmin	Diagnosticiranje glioma
Spanish	Tetrofosmina	Diagnóstico del glioma
Swedish	Tetrofosmin	Diagnos av gliom
Norwegian	Tetrofosmin	Diagnose av gliom
Icelandic	Tetófosmín	Sjúkdómsgreining glióma

[1] At the time of designation