



Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation

of 4-^[123I] iodo-L-phenylalanine for the diagnosis of glioma

On 25 July 2006, orphan designation (EU/3/06/386) was granted by the European Commission to Dr Andreas Kluge, Germany, for 4-^[123I] iodo-L-phenylalanine for the diagnosis of glioma. The sponsorship was transferred to Therapiea GmbH & Co. KG, Germany, in February 2008.

What is glioma?

Glioma is a type of brain tumour that begins in 'glial' cells (the cells that surround and support nerve cells). Patients with gliomas can suffer from different symptoms, depending on the part of the brain where the tumour develops. Glioma is a life-threatening disease.

What is the estimated number of patients at risk of developing the condition?

At the time of designation gliomas affected 1.5 in 10,000 people in the European Union (EU) *. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP). This is below the threshold for orphan designation which is 5 in 10,000. This is equivalent to a total of around 70,000 people.

What are the methods of diagnosis available?

Diagnosis for glioma depends on a number of factors and encompasses several methods using different equipment. Diagnosis includes certain contrast agents used for magnetic resonance imaging. Different methods to diagnose gliomas were authorised in the Community at the time of submission of the application for orphan designation.

Satisfactory argumentation has been submitted by the sponsor to justify that 4-^[123I] iodo-L-phenylalanine might be of potential significant benefit for the diagnosis of glioma because it might improve the diagnosis of the patients as it can be taken up by malignant glioma cells and retained in the tumours for up to 24 hours. These features may allow improved visualisation and diagnosis of gliomas. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicinal product expected to work?

The product is a radioactive element (^{123I}) attached to an amino acid, L-phenylalanine. Iodine^[123I] can give off radiation that can be detected by imaging methods from outside the body. It is expected that 4-^[123I] iodo-L-phenylalanine accumulates in the glioma cancer cells and help visualising the tumour tissue with the radiation produced by the attached radioactive iodine.

* Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 25), Norway, Iceland and Liechtenstein. This represents a population of 459,700,000 (Eurostat 2004).

What is the stage of development of this medicinal product?

The evaluation of the effects of 4-[¹²³I] iodo-L-phenylalanine in experimental models was ongoing.

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

4-[¹²³I] iodo-L-phenylalanine was not authorised anywhere worldwide for glioma or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 15 June 2006 a positive opinion recommending the grant of the above-mentioned 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;
- and either the rarity of the condition (affecting not more than five in 10,000 people in the Community) or the 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 the quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

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**Translations of the active ingredient and indication in all EU languages
and Norwegian and Icelandic**

Language	Active Ingredient	Indication
English	4- ¹²³ Ijiodo-L-phenylalanine	Diagnosis of glioma
Czech	4- ¹²³ Ijód-L-fenylalanin	Diagnóza gliomu
Danish	4- ¹²³ Ijiod-L-phenylalanin	Diagnose af gliom
Dutch	4- ¹²³ Ijiodium-L-phenylalanine	Diagnose van glioma
Estonian	4- ¹²³ Ijiood-L-fenüüalaniniin	Glioomi diagnoosimiseks
Finnish	4- ¹²³ Ijiodo-L-fenylalaniini	Gliooman diagnosointi
French	4- ¹²³ Ijiodo-L-phénylalanine	Diagnostic des gliomes
German	4- ¹²³ IjIod-L-Phenylalanin	Diagnose des Glioms
Greek	4- ¹²³ Ijίω L δο – L – Φαινυλαλανίνη	διάγνωση του γλειώματος
Hungarian	4- ¹²³ Ijód-L-phenylalanin	Glioma diagnosztizálása
Italian	4- ¹²³ Ijiodo-L-fenilalanina	Diagnosi del glioma
Latvian	4- ¹²³ Ijiod-L-fenilalanīns	Gliomas diagnostika
Lithuanian	4- ¹²³ Ijiodo-L-fenilalaninas	Gliomos diagnozė
Polish	4- ¹²³ Ijiodo L- fenyloalanina	Rozpoznanie glejaka
Portuguese	4- ¹²³ Ijiodo-L-fenilalanina	Diagnóstico do glioma
Slovak	4- ¹²³ Ijód-L-fenylalanín	Diagnóza gliómu
Slovenian	4- ¹²³ Ijiodo-L-fenilalanin	Diagnosticiranje glioma
Spanish	4- ¹²³ Ijiodo-L-fenilalanina	Diagnóstico del glioma
Swedish	4- ¹²³ Ijiod-L-fenylalanin	Diagnos av gliom
Norwegian	4- ¹²³ Ijiod-L-fenylalanin	Diagnose av gliom
Icelandic	4- ¹²³ Ijjoð-L- fenýlalanín	Sjúkdómsgreining glioma