



European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of 4-[¹³¹I] iodo-L-phenylalanine for the treatment of glioma

On 11 April 2006, orphan designation (EU/3/06/363) was granted by the European Commission to Dr Andreas Kluge, Germany, for 4-[¹³¹I] iodo-L-phenylalanine for the treatment 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 affected by the condition?

At the time of designation glioma affected approximately 1 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 46,000 persons.

What treatments are available?

Treatment for glioma may combine different methods such as surgery, radiotherapy (treatment with radiation) and chemotherapy (medicines used to treat cancer). Other treatments are used to relieve the disease's symptoms, including corticosteroids that help to reduce pressure within the skull, and medicines that help with the control of seizures (fits). At the time of orphan designation, several medicines were authorised in the European Union for the treatment of glioma and its symptoms. 4-[¹³¹I] iodo-L-phenylalanine might be of potential significant benefit for the treatment of glioma because it might improve the long-term outcome of the patients. The 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 has a radioactive element (iodine 131) attached to an amino acid, L-phenylalanine. Iodine-131 can give off radiation that can damage and kill cells, especially those that are dividing rapidly, such as cancer cells. Thus, it is expected that 4-[¹³¹I] iodo-L-phenylalanine will accumulate in the glioma cancer cells, and kill them with the radiation produced by the attached iodine 131.

What is the stage of development of this medicinal product?

The evaluation of the effects of 4-[¹³¹I] iodo-L-phenylalanine in experimental models was ongoing.

* 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).

At the time of submission of the application for orphan designation, no clinical trials in patients with glioma were initiated.

4-^[131]I] 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 8 March 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-[¹³¹ I]iodo-L-phenylalanine	Treatment of glioma
Czech	4-[¹³¹ I]jód-L-fenylalanin	Léčba gliomů
Danish	4-[¹³¹ I]iod-L-phenylalanin	Behandling af gliom
Dutch	4-[¹³¹ I]jodium-L-phenylalanine	Behandeling van glioma
Estonian	4-[¹³¹ I]iood-L-fenüüalaniniin	Glioomi ravi
Finnish	4-[¹³¹ I]iodo-L-fenylalaniini	Gliooman hoito
French	4-[¹³¹ I]iodo-L-phénylalanine	Traitement des gliomes
German	4-[¹³¹ I]Iod-L-Phenylalanin	Behandlung von Gliomen
Greek	4-[¹³¹ I]ιω L δο – L – Φαινυλαλανίνη	Θεραπεία του γλοιώματος
Hungarian	4-[¹³¹ I]jód-L-phenylalanin	Glioma kezelése
Italian	4-[¹³¹ I]iodo-L-fenilalanina	Trattamento del glioma
Latvian	4-[¹³¹ I]jod-L-fenilalanīns	Gliomas ārstēšana
Lithuanian	4-[¹³¹ I] jodo-L-fenilalaninas	Gliomos gydymas
Polish	4-[¹³¹ I]jodo L- fenyloalanina	Leczenie glejaka
Portuguese	4-[¹³¹ I]iodo-L-fenilalanina	Tratamento do glioma
Slovak	4-[¹³¹ I]jód-L-fenylalanín	Liečba gliómu
Slovenian	4-[¹³¹ I] jodo-L-fenilalanin	Zdravljenje glioma
Spanish	4-[¹³¹ I]yodo-L-fenilalanina	Tratamiento del glioma
Swedish	4-[¹³¹ I]jod-L-fenylalanin	Behandling av gliom
Norwegian	4-[¹³¹ I]iod-L-fenylalanin	Behandling av gliom
Icelandic	4-[¹³¹ I]joð-L- fenýlalanín	Meðhöndlun á glióma