

1 September 2011 EMA/COMP/467543/2007 Rev.3 Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Iodine (¹³¹I) chlorotoxin for the for the treatment of glioma

On 22 October 2007, orphan designation (EU/3/07/492) was granted by the European Commission to the Weinberg Group LLC, United Kingdom, for iodine (¹³¹I) chlorotoxin for the treatment of glioma.

The sponsorship was transferred to The Weinberg Group Limited, United Kingdom, in March 2009, to Biological Consulting Europe Ltd, United Kingdom, in December 2010 and subsequently to Eisai Europe Limited, in June 2011.

What is glioma?

Tumours that begin in brain tissue are known as primary brain tumours. Primary brain tumours are named after the type of tissue from which they develop, the most common being gliomas, which begin in the glial (supportive) tissue. Patients affected by gliomas can suffer from severe symptoms of the nervous system, depending on where in the brain the tumour develops. Glioma is life-threatening.

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 equivalent to a total of around 50,000 people, and is below the threshold 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 treatments are available?

Treatment for glioma depends on a number of factors and includes different methods such as surgery, radiotherapy (using high-dose x-rays or other high-energy rays to kill cancer cells) or chemotherapy (using drugs to kill cancer cells), as well as some symptomatic treatments. Symptomatic treatments include certain steroid hormones (corticosteroids) to control the effects of raised pressure within the skull, and medication to help control seizures, as required. Several products for the treatment of glioma were authorised in the Community at the time of submission of the application for orphan designation.

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^{*}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 27), Norway, Iceland and Liechtenstein. This represents a population of 498,000,000 (Eurostat 2006).

Iodine (¹³¹I) chlorotoxin 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 medicine expected to work?

The product is a radioactive element (iodine 131) attached to a protein, which specifically binds to glioma cells. Iodine-131 can give off radiation, which in turn damages and kills cells, especially those that are dividing, such as cancer cells. Thus, it is expected that iodine (¹³¹I) chlorotoxin will build up in the glioma cancer cells, and then kill these cells with the radiation produced by the radioactive iodine contained in the molecule.

What is the stage of development of this medicine?

The effects of Iodine (¹³¹I) chlorotoxin were evaluated in experimental models.

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

Iodine (¹³¹I) chlorotoxin was not authorised anywhere in the world for glioma, at the time of submission. Orphan designation of Iodine (¹³¹I) chlorotoxin was granted in the United States for the treatment of malignant glioma.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 September 2007 recommending the granting of this 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;
- 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:

Eisai Europe Limited European Knowledge Centre Mosquito Way Hatfield Herts AL10 9SN United Kingdom Telephone: +44 (0) 845 676 5089 Telefax: +44 (0) 845 676 1504

For contact details of patients' organisations whose activities are targeted at rare diseases see:

- <u>Orphanet</u>, a database containing information on rare diseases which includes a directory of patients' organisations registered in Europe.
- <u>European Organisation for Rare Diseases (EURORDIS)</u>, 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 languages¹, Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Iodine (¹³¹ I) chlorotoxin	Treatment of glioma
Bulgarian	Йод (¹³¹ I) хлоротоксин	Лечение на глиома
Czech	Jód 131 chlorotoxin	Léčba gliomů
Danish	¹³¹ I(Jod-131) chlorotoxin	Behandling af gliom
Dutch	Iodium ¹³¹ chlorotoxine	Behandeling van glioma
Estonian	Jood (¹³¹ I) klorotoksiin	Glioomi ravi
Finnish	Jodi (¹³¹ I) klorotoksiini	Gliooman hoito
French	Chlorotoxine marquée à l'iode ¹³¹ I	Traitement des gliomes
German	Jod (I-131) chlorotoxin	Behandlung von Glioma
Greek	Ιωδιο (131Ι)- χλωροτοξίνη	Θεραπεία του γλοιώματος
Hungarian	[¹³¹ I]jód- klorotoxin	Glioma kezelése
Italian	¹³¹ I -clorotossina	Trattamento del glioma
Latvian	Joda (¹³¹ I) hlorotoksīns	Gliomas ārstēšana
Lithuanian	Jodo (¹³¹ I) chlorotoksinas	Gliomos gydymas
Maltese	Iodine (¹³¹ I) chlorotoxin	Kura tal-glioma
Polish	Jodowana (¹³¹ I) chlorotoksyna	Leczenie glejaka
Portuguese	Clorotoxina com Iodo (¹³¹ I)	Tratamento do glioma
Romanian	Clorotoxină de Iod (¹³¹ I)	Tratamentul gliomului
Slovak	Jód (¹³¹ I) chlorotoxín	Liečba gliómu
Slovenian	Jodov (¹³¹ I) klorotoksin	Zdravljenje glioma
Spanish	Clorotoxina marcada con Yodo (¹³¹ I)	Tratamiento del glioma
Swedish	Jod(¹³¹ I)klorotoxin	Behandling av gliom
Norwegian	Jod (1311) klortoksin	Behandling av gliom
Icelandic	Joð (1311) klórótoxín	Meðhöndlun á glíóma

¹ At the time of designation