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 gimatecan for the treatment of glioma

On 1 December 2003, orphan designation (EU/3/03/174) was granted by the European Commission to Sigma Tau Industrie Farmaceutiche Riunite S.p.A, Italy, for gimatecan for the treatment of glioma. The sponsorship was transferred to Novartis Europharm Limited, United Kingdom, in November 2005 and subsequently to Sigma-Tau Industrie Farmaceutiche Riunite S.p.A., Italy, in May 2009.

What is glioma?
Tumours that begin in brain tissue are known as primary brain tumours. Primary brain tumours are classified by the type of tissue from which they originate, the most common being gliomas, which begin in the glial (supportive) tissue.
Gliomas represent a potentially debilitating and life-threatening condition, with symptoms being influenced by which regions of the brain are affected. Patients affected by gliomas can suffer from medical problems to the nervous system, depending on where in the brain the tumour develops.

What is the estimated number of patients affected by the condition?
At the time of designation, glioma affected approximately 0.8 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 31,000 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 knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?
Treatment for gliomas depends on a number of factors and may include 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 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. Methods of treatment of glioma were authorised at the time of submission of the application for orphan designation.
Gimatecan might be of potential significant benefit for the treatment of gliomas. The main reason is that gimatecan may offer a new way of killing the cancer cells in these patients. The assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

[*]Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of about 384,000,000 (Eurostat 2002) and may differ from the true number of patients affected by the condition.

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How is this medicine expected to work?
Gimatecan is a drug that inhibits an enzyme needed for cell division. This results in inhibition of cell division and tumour growth.

What is the stage of development of this medicine?
At the time of submission of the application for orphan designation, clinical trials in patients with glioma were ongoing.

Gimatecan was not marketed anywhere worldwide for glioma, at the time of submission. Orphan designation of gimatecan was granted in the United States for malignant glioma.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 10 October 2003 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;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the Community) 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.

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Translations of the active ingredient and indication in all EU languages

Language	**Active Ingredient**	**Indication**
English	Gimatecan	Treatment of glioma
Danish	Gimatecan	Behandling af gliom
Dutch	Gimatecan	Behandeling van glioma
Finnish	Gimatecan	Gliooman hoito
French	Gimatécan	Traitement des gliomes
German	Gimatecan	Behandlung des Glioms
Greek	Γιματεκάνη	Θεραπεία του γλοιώματος
Italian	Gimatecan	Trattamento del glioma
Portuguese	Gimatecan	Tratamento do glioma
Spanish	Gimatecán	Tratamiento del glioma
Swedish	Gimatecan	Behandling av gliom