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 enzastaurin hydrochloride for the treatment of glioma

On 23 December 2005, orphan designation (EU/3/05/343) was granted by the European Commission to Eli Lilly Nederland B.V., The Netherlands, for enzastaurin hydrochloride for the treatment of glioma.

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. Patients affected by gliomas can suffer from severe symptoms of the nervous system, depending on where in the brain the tumour develops.

What are the methods of treatment available?

Treatment for gliomas depends on a number of factors and encompasses several 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. Methods of treatment of glioma were authorised in the Community at the time of submission of the application for orphan designation.
Enzastaurin hydrochloride might be of potential significant benefit for the treatment of gliomas 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.

What is the estimated number of patients affected by the condition*?

According to the information provided by the sponsor, glioma was considered to affect about 46,000 persons in the European Union.

How is this medicinal product expected to act?

Cancer cells need to replicate and produce a network of new blood vessels in order to grow. Enzastaurin hydrochloride blocks (inhibits) the function of certain proteins such as protein kinase pathways that have been shown to be involved in the progression of some types of cancer, including glioma. The theory is that enzastaurin hydrochloride, through its interference with the function of these proteins, will prevent the glioma from growing by preventing tumour cell replication and development of new blood vessels and by inducing tumour cell death.

What is the stage of development of this medicinal product?

The evaluation of the effects of enzastaurin hydrochloride in experimental models is ongoing.

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

Enzastaurin hydrochloride 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 10 November 2005 a positive opinion recommending the grant of the above-mentioned designation.

Opinions on orphan medicinal products designations are based on the following cumulative criteria: (i) the seriousness of the condition, (ii) the existence or not of alternative methods of diagnosis, prevention or treatment and (iii) either the rarity of the condition (considered to affect not more than five in ten thousand persons in the Community) or the insufficient return of development investments.

Designated orphan medicinal products are still investigational products which were considered for 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 will be necessary before this product can be granted a marketing authorisation.

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*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 Lichtenstein. This represents a population of 459,700,000 (Eurostat 2004). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

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

Language	Active Ingredient	Indication
English	Enzastaurin hydrochloride	Treatment of glioma
Czech	Hydrochlorid enzastaurinum	Léčba gliomů
Danish	Enzastaurin hydrochlorid	Behandling af gliom
Dutch	Enzastaurine hydrochloride	Behandeling van glioma
Estonian	Entsastauriinhüdrokloriid	Glioomi ravi
Finnish	Entsastauriinin hydrokloridi	Gliooman hoito
French	Hydrochloride de enzastaurine	Traitement des gliomes
German	Enzastaurinhydrochlorid	Behandlung des Glioms
Greek	Ενζασταυρίνη υδροχλωρική	Θεραπεία του γλοιώματος
Hungarian	Enzastaurin hidroklorid	Glioma kezelése
Italian	Enzastaurin idrocloruro	Trattamento del glioma
Latvian	Enzastaurīns hidrohlorīds	Gliomas ārstēšana
Lithuanian	Enzastaurinas hidrochloridas	Gliomos gydymas
Polish	Enzastauryny chlorowodorek	Leczenie glejaka
Portuguese	Cloridrato de enzastaurina	Tratamento do glioma
Slovak	Enzastaurin hydrochlorid	Liečba gliómu
Slovenian	Enzastaurin hidroklorid	Zdravljenje glioma
Spanish	Clorhidrato de enzastaurina	Tratamiento del glioma
Swedish	Enzastaurin hydroklorid	Behandling av gliom
Norwegian	Enzastaurinhydroklorid	Behandling av gliom
Icelandic	Enzastaurin hýdróklóríð	Meðhöndlun á glíóma