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 irinotecan hydrochloride (drug eluting beads) for the treatment of glioma

On 29 November 2007, orphan designation (EU/3/07/504) was granted by the European Commission to CellMed AG, Germany, for irinotecan hydrochloride (drug eluting beads) for the treatment of glioma.

What are gliomas?

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 originate. The most common brain tumours are gliomas, which begin in the glial (supportive) tissue. Due to their location, 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. Gliomas are life-threatening.

What are the methods of treatment available?

Treatment of gliomas depends on a number of factors and may include surgery, radiotherapy or chemotherapy as well as symptomatic treatments, such as corticosteroids to control the effects of raised pressure within the skull and medication to help control seizures, as required. Several medicinal products for the treatment of the condition were authorised at the time of submission of the application for orphan designation. Satisfactory argumentation has been submitted by the sponsor to justify the assumption that irinotecan hydrochloride (drug eluting beads) might be of potential significant benefit for the treatment of glioma, particularly in terms of its new route of administration.
This 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*?

Based on the information provided by the sponsor and previous knowledge of the Committee, gliomas were considered to affect approximately 1 in 10,000 persons in the European Union, which, at the time of designation, corresponded to about 50,000 persons.

*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 27), Norway, Iceland and Lichtenstein. This represents a population of 498,000,000 (Eurostat 2006). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

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How is this medicinal product expected to act?
Irinotecan hydrochloride (drug eluting beads) is going to be given directly into the brain, by injection into the resection margin of the tumour. Therefore the chemotherapeutic agent (irinotecan hydrochloride) could be released from the drug eluting beads directly into the tumour. As a consequence irinotecan hydrochloride (drug eluting beads) may maximize the dose of irinotecan hydrochloride to the tumour whilst decreasing the systemic side effects due to lower blood levels of irinotecan hydrochloride.

What is the stage of development of this medicinal product?
The effects of irinotecan hydrochloride (drug eluting beads) were evaluated in experimental models.

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

Irinotecan hydrochloride (drug eluting beads) was not authorised anywhere worldwide for the treatment of 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 October 2007 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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Translations of the active ingredient and indication in all EU languages and Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Irinotecan hydrochloride (drug eluting beads)	Treatment of glioma
Bulgarian	Иринотекан хидрохлорид (микросфери, освобождаващи лекарствено вещество)	Лечение на глиома
Czech	Irinotekan hydrochlorid (kuličky uvolňující lék)	Léčba gliomů
Danish	Irinotecanhydroklorid (medicineluerende kugler)	Behandling af gliom
Dutch	Irinotecanhydrochloride (geneesmiddel eluerende parels)	Behandeling van glioma
Estonian	Irinotekaanvesinikkloriid (ravim sisaldub organismis lagunevates kapslites)	Glioomi ravi
Finnish	Irinotekaanihydrokloridi (lääkettä uuttavat helmet)	Gliooman hoito
French	Chlorhydrate d'rinotécan (perles a elution medicamenteuse)	Traitement des gliomes
German	Irinotecanhydrochlorid (Medikament freisetzende Perlen)	Behandlung von Gliomen
Greek	Υδροχλωρική Ιρινοτεκάνη (σφαιριδια εκλουσης φαρμακου)	Θεραπεία του γλοιώματος
Hungarian	Irinotecan-hidroklorid (hatóanyagleadó mikropelletek)	Glioma kezelése
Italian	Irinotecan cloridrato (microsfere ad eluizione di farmaco)	Trattamento del glioma
Latvian	Irinotekāna hidrohlorīds (zāles izdalošas lodītes)	Gliomas ārstēšana
Lithuanian	Irinotekano hidrochloridas (mikrosferos, įsotintos vaistais)	Gliomos gydymas
Maltese	Irinotecan hydrochloride (żibeġ għall-elużjoni tal-mediċina)	Kura tal-glioma
Polish	Irynotekan chlorowodorek (granulkiuwalniające lek)	Leczenie glejaka
Portuguese	Cloridrato de irinotecan (esferas de eluição medicamentosas)	Tratamento do glioma
Romanian	Clorhidrat de irinotecan (microsfere eliberatoare de medicament)	Tratamentul gliomului
Slovak	Irinotekániumchlorid (mikrokapsuly uvoľňujúce liek)	Liečba gliómu
Slovenian	Irinotecán hidroklorid (eluacijska polnila za zdravilo)	Zdravljenje glioma
Spanish	Clorhidrato de irinotecán (cuentas liberadoras de medicamento)	Tratamiento del glioma
Swedish	Irinotecanhydroklorid (läkemedelsutsöndrande pärlor)	Behandling av gliom
Norwegian	Irinotecanhydroklorid (legemiddelutskillende kuler)	Behandling av gliom
Icelandic	Írinótekan hýdróklóríð (perluband sem skolar út lyfi úr kúlunum)	Meðhöndlun á glióma