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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

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

On 29 November 2007, orphan designation (EU/3/07/507) was granted by the European Commission to CellMed Agf, Germany, for doxorubicin hydrochloride (drug eluting beads) for the treatment of glioma.

The sponsorship was transferred to Biocompatibles UK Limited, United Kingdom, in October 2012.

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 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. 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 was equivalent to a total of around 50,000 people*, and is below the ceiling 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 may include surgery, radiotherapy or chemotherapy as well as symptomatic treatments, such as corticosteroids to control the effects of the 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

*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.
At the time of designation, this represented a population of 500,300,000 (Eurostat 2007).

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assumption that doxorubicin hydrochloride (drug eluting beads) might be of potential significant benefit for the treatment of glioma, particularly in terms of its new form and 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.

How is this medicine expected to work?

Doxorubicin hydrochloride (drug eluting beads) is going to be administered directly into the brain, by injection into the resection margins of the tumour. Therefore, the chemotherapeutic agent (doxorubicin hydrochloride) could be released from the drug eluting beads directly into any possible tumour residue. As a consequence doxorubicin hydrochloride (drug eluting beads) may increase the dose of doxorubicin hydrochloride to the tumour residue, whilst decreasing the side effects due to lower levels of doxorubicin hydrochloride in the blood.

What is the stage of development of this medicine?

The effects of doxorubicin 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 had been initiated.

Doxorubicin hydrochloride (drug eluting beads) was not authorised anywhere in the world for the treatment of glioma or designated as an orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 10 October 2007 recommending the granting of this designation.

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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:

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

- Orphanet, a database containing information on rare diseases which includes a directory of patients' organisations registered in Europe.
- European Organisation for Rare Diseases (EURORDIS), 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 languages[1], Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Doxorubicin hydrochloride (drug eluting beads)	Treatment of glioma
Bulgarian	Доксорубицин хидрохлорид (микросфери, освобождаващи лекарствено вещество)	Лечение на глиома
Czech	Doxorubicin hydrochlorid (kuličky uvolňující lék)	Léčba gliomů
Danish	Doxorubicinhydroklorid (medicineluerende kugler)	Behandling af gliom
Dutch	Doxorubicinehydrochloride (geneesmiddel eluerende parels)	Behandeling van glioma
Estonian	Doksorubitsiinvesinikkloriid (ravim sisaldub organismis lagunevates kapslites)	Glioomi ravi
Finnish	Doksorubisiinihydroklorid (lääkettä uuttavat helmet)	Glioman hoito
French	Chlorhydrate de doxorubicine (perles a elution medicamenteuse)	Traitement des gliomes
German	Doxorubicinhydrochlorid (Medikament freisetzende Perlen)	Behandlung von Gliomen
Greek	Υδροχλωρική δοξορουβικίνη (σφαιρίδια έκλουσης φαρμάκου)	Θεραπεία του γλοιώματος
Hungarian	Doxorubicin-hidroklorid (hatóanyagleadó mikropelletek)	Glioma kezelése
Italian	Doxorubicina cloridrato (microsfere ad eluizione di farmaco)	Trattamento del glioma
Latvian	Doksorubicīna hidrohlorīds (zāles izdalošas lodītes)	Gliomas ārstēšana
Lithuanian	Doksorubicino hidrochloridas (mikrosferos, įsotintos vaistais)	Gliomos gydymas
Maltese	Doxorubicin hydrochloride (żibeġ għall-elużjoni tal-mediċina)	Kura tal-glioma
Polish	Doksorubicyny chlorowodorek (mikrokapsułki uwalniające lek)	Leczenie glejaka
Portuguese	Cloridrato de doxorrubicina (esferas de eluiçãomedicamentosas)	Tratamento do glioma
Romanian	Clorhidrat de doxorubicină (microsfere eliberatoare de medicament)	Tratamentul gliomului
Slovak	Doxorubicíniumchlorid (mikrokapsuly uvoľňujúce liek)	Liečba gliómu
Slovenian	Doksorubicin hidroklorid (eluacijska polnila za zdravilo)	Zdravljenje glioma
Spanish	Doxorubicina clorhidrato (cuentas liberadoras de medicamento)	Tratamiento del glioma

[1] At the time of designation

Language	Active Ingredient	Indication
Swedish	Doxorubicinhydroklorid (läkemedelsutsöndrande pärlor)	Behandling av gliom
Norwegian	Doksorubicinhydroklorid (legemiddelutskillende kuler)	Behandling av gliom
Icelandic	Doxórúbicín hýdróklóríð (perluband sem skolar út lyfi úr kúlunum)	Meðhöndlun á glíóma