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 gadodiamide (liposomal) for the adjunctive treatment of glioma

On 3 December 2008, orphan designation (EU/3/08/583) was granted by the European Commission to Dr. Matthias Luz, Germany, for gadodiamide (liposomal) for the adjunctive treatment of glioma.

What is glioma?

Glioma is a type of brain tumour that affects the glial cells (the cells that support the nerve cells). Patients with glioma can have severe symptoms, but the types of symptoms experienced depend on where the tumour develops in the brain. Symptoms can include headaches, nausea, loss of appetite, vomiting, and changes in personality, mood, mental capacity and concentration. About 20% of patients have fits before the glioma is diagnosed. Glioma is a life-threatening disease.

What is the estimated number of patients affected by glioma?

At the time of designation, glioma affected approximately 1 in 10,000 people in the European Union (EU)[*]. This is below the threshold for orphan designation which is 5 in 10,000, and is equivalent to a total of around 50,000 people. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

Several types of treatment for glioma may be used, such as surgery, radiotherapy (using radiation to kill cancer cells) or chemotherapy (medicines to treat cancer). Treatments to relieve symptoms can also be used.

Gadodiamide (liposomal) is expected to improve the precision with which certain anticancer medicines are used in the treatment of glioma, by enabling the doctor to see where the medicine is distributed in the body. This may improve the efficacy and safety of the anticancer medicine. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Gadodiamide (liposomal) is made up by encapsulating a substance called gadodiamide in small fat particles called 'liposomes'. Gadodiamide[1] is a chemical that contains the metal gadolinium and which is already authorised as an imaging agent to help obtain better images of the inside of the body with MRI scans. MRI is an imaging method that uses magnetic fields and radio waves. Because the gadodiamide is contained in liposomes, it can cross the 'blood-brain barrier' that separates the bloodstream from the brain tissue. This means that, unlike 'free' gadodiamide, it can get into the brain.

[*] 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 Liechtenstein. This represents a population of 502,282,000 (Eurostat 2008).

[1] Gadodiamide is available in the EU under the trade name Omniscan.

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Gadodiamide (liposomal) has been developed to be used together with the anticancer medicine topotecan. During the MRI scan, gadodiamide (liposomal) is used to track where the topotecan goes within the brain. Gadodiamide (liposomal) is expected to show how much of the tumour the medicine has reached and to indicate where more treatment may be needed. It is also expected to help to see that any catheters (small tubes inserted into the veins) are correctly placed.

What is the stage of development of this medicine?
The evaluation of the effects of gadodiamide (liposomal) in experimental models was ongoing.

At the time of submission of the application for orphan designation, clinical trials in patients with glioma had not started.

At the time of submission, gadodiamide (liposomal) was not authorised anywhere in the world for glioma or designated as orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 October 2008 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 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 marketing authorisation.

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Patients' association(s) contact point(s): Pending

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

Language	Active ingredient	Indication
English	Gadodiamide (liposomal)	Treatment of glioma
Bulgarian	Gadodiamide (липозомален)	Лечение на глиома
Czech	Gadodiamide (liposomální)	Léčba gliomů
Danish	Gadodiamid (liposomalt)	Behandling af gliom
Dutch	Gadodiamide (liposomaal)	Behandeling van glioma
Estonian	Gadodiamiid (liposomaalne)	Glioomi ravi
Finnish	Gadodiamidi (liposomaalinen)	Gliooman hoito
French	Gadodiamide (liposomal)	Traitement des gliomes
German	Gadodiamid (liposomal)	Behandlung von Gliomen
Greek	Gadodiamide (Λιποσωμική)	Θεραπεία του γλοιώματος
Hungarian	Gadodiamide (liposzómában)	Glioma kezelése
Italian	Gadodiamide (liposomiale)	Trattamento del glioma
Latvian	Gadodiamīds (liposomu)	Gliomas ārstēšana
Lithuanian	Gadodiamidas (liposominis)	Gliomos gydymas
Maltese	Gadodiamide (liposomali)	Kura tal-glioma
Polish	Gadodiamid (liposomalny)	Leczenie glejaka
Portuguese	Gadodiamida (liposomal)	Tratamento do glioma
Romanian	Gadodiamidă (inclusă în lipozomi)	Tratamentul gliomului
Slovak	Gadodiamid (lipozomálny)	Liečba gliómu
Slovenian	Gadodiamid (liposomski)	Zdravljenje glioma
Spanish	Gadodiamida (liposomal)	Tratamiento del glioma
Swedish	Gadodiamid (liposomal)	Behandling av gliom
Norwegian	Gadodiamid (liposomalt)	Behandling av gliom
Icelandic	Gadódíamíð (í lípósómum)	Meðferð á glíóma