



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Public summary of opinion on orphan designation

Combination of carboplatin and sodium valproate for the treatment of glioma

On 31 July 2018, orphan designation (EU/3/18/2043) was granted by the European Commission to Dr Ulrich Granzer, Germany, for combination of carboplatin and sodium valproate for the treatment of glioma.

What is glioma?

Glioma is a type of brain tumour that affects the 'glial' cells (the cells that surround and support the nerve cells). Patients with glioma can have severe symptoms, but the types of symptoms depend on where the tumour develops in the brain.

Symptoms can include headaches, nausea (feeling sick), loss of appetite, vomiting, and changes in personality, mood, mental capacity and concentration. About one-fifth of patients with glioma have seizures (fits) for months or years before the disease is diagnosed.

Glioma is a long-term debilitating and life-threatening disease because of the severe damage to the brain, and it is associated with poor long-term survival.

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

At the time of designation, glioma affected approximately 2.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 135,000 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?

At the time of designation, several medicines were authorised for the treatment of glioma in the EU. Treatments for glioma included radiotherapy (treatment with radiation), chemotherapy (medicines to treat cancer) and surgery. Patients also received treatments for the symptoms of glioma, including corticosteroids to reduce pressure within the skull and medicines to prevent seizures.

^{*}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 28), Norway, Iceland and Liechtenstein. This represents a population of 517,400,000 (Eurostat 2018).



The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with glioma because early data showed that patients treated with this medicine lived longer when compared indirectly with results seen with authorised treatments. 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?

This medicine is a combination of the cancer medicine carboplatin and the epilepsy medicine sodium valproate, both of which have been used for many years in the EU for other conditions.

Carboplatin belongs to a group of medicines called alkylating agents. Alkylating agents are highly reactive chemicals that attach to genetic material in the cell, and can damage or kill the cell.

In glioma, sodium valproate is expected to have several actions, including keeping the genes that suppress the growth of the cancer cells switched 'on'. This is expected to slow down the growth of the cancer.

The medicines are expected to be delivered directly into the brain from a special device implanted at the back of the head.

What is the stage of development of this medicine?

The effects of carboplatin and sodium valproate alone into the brain have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the combination in patients with glioma had been started.

At the time of submission, the combination was not authorised anywhere in the EU for glioma or designated as an 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 21 June 2018 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 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's [rare disease designations page](#).

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 languages¹, Norwegian and Icelandic

Language	Activeingredient	Indication
English	Combination of carboplatin and sodium valproate	Treatment of glioma
Bulgarian	Комбинация от карбоплатин и натриев валпроат	Лечение на глиома
Croatian	Kombinacija karboplatina i natrijevog valproata	Liječenje glioma
Czech	Kombinace karboplatiny a valproátu sodného	Léčba gliomů
Danish	Kombination af carboplatin og natriumvalproat	Behandling af gliom
Dutch	Combinatie van carboplatine en natriumvalproaat	Behandeling van glioma
Estonian	Karboplatiini ja naatriumvalproaadi kombinatsioon	Glioomi ravi
Finnish	Karboplatiinin ja natriumvalproaatin yhdistelmä	Gliooman hoito
French	Association de carboplatine et de valproate de sodium	Traitement des gliomes
German	Kombination aus Carboplatin und Natriumvalproat	Behandlung von Gliomen
Greek	Συνδυασμός καρβοπλατίνης και βαλπροϊκού νατρίου	Θεραπεία του γλοιώματος
Hungarian	Karboplatin és nátrium-valproát kombinációja	Glioma kezelése
Italian	Combinazione di carboplatino e sodio valproato	Trattamento del glioma
Latvian	Karboplatīna un nātrija valproāta kombinācija	Gliomas ārstēšana
Lithuanian	Karboplatinos ir natrio valproato derinys	Gliomos gydymas
Maltese	Taħlita ta' karboplatin u valproat tas-sodju	Kura tal-glioma
Polish	Połączenie karboplatyny z walproinianem sodu	Leczenie glejaka
Portuguese	Associação de carboplatina e valproato de sódio	Tratamento do glioma
Romanian	Combinație de carboplatină și valproat de sodiu	Tratamentul gliomului
Slovak	Kombinácia karboplatiny a valproátu sodného	Liečba gliómu
Slovenian	Kombinacija kaboplatina in natrijevega valproata	Zdravljenje glioma
Spanish	Combinación de carboplatino y valproato de sodio	Tratamiento del glioma
Swedish	kombination av karboplatin och natriumvalproat	Behandling av gliom
Norwegian	Kombinasjon av karboplatin og natriumvalproat	Behandling av gliom
Icelandic	Samsetning karbóplatíns og natríumvalpróats	Meðferð á glíóma

¹ At the time of designation