



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Eflornithine for the treatment of glioma

On 27 June 2016, orphan designation (EU/3/16/1679) was granted by the European Commission to Orbus Therapeutics Limited, United Kingdom, for eflornithine 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 experienced depend on where the tumour develops in the brain.

Symptoms can include headaches, nausea, vomiting, loss of appetite 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 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 134,000 people<sup>\*</sup>, 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 surgery, radiotherapy (treatment with radiation), and chemotherapy (medicines to treat cancer) to improve survival. Patients also received treatments for the symptoms of glioma, including corticosteroids to reduce pressure within the skull and medicines to prevent seizures.

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<sup>\*</sup>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 513,700,000 (Eurostat 2016).



The sponsor has provided sufficient information to show that eflornithine might be of significant benefit for patients with glioma because early studies indicate that it may improve survival of patients when used in combination with other authorised medicines. 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?**

Eflornithine works by blocking the action of an enzyme called ornithine decarboxylase, which is involved in the production of polyamines, substances required for cells to grow. In some cancers including glioma, the activity of polyamine enzymes has been linked with the rapid growth of cancer cells. By blocking ornithine decarboxylase, eflornithine is expected to slow down the growth of the glioma cells.

### **What is the stage of development of this medicine?**

The effects of eflornithine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with eflornithine in patients with glioma had been completed.

At the time of submission, eflornithine was authorised in the EU and United states for the treatment of facial hirsutism in women.

At the time of submission, eflornithine was not authorised anywhere in the EU for glioma. Orphan designation of this medicine had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 19 May 2016 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:

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 languages<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Eflornithine	Treatment of glioma
Bulgarian	Ефлорнитин	Лечение на глиома
Croatian	Eflornitin	Liječenje glioma
Czech	Eflornithin	Léčba gliomů
Danish	Eflornithin	Behandling af gliom
Dutch	Eflornithine	Behandeling van glioma
Estonian	Eflornitiin	Glioomi ravi
Finnish	Eflornitiini	Gliooman hoito
French	Eflornithine	Traitement des gliomes
German	Eflornithin	Behandlung von Gliomen
Greek	Εφλορνιθίνη	Θεραπεία του γλοιώματος
Hungarian	Eflornitin	Glioma kezelése
Italian	Eflornitina	Trattamento del glioma
Latvian	Eflornitiņš	Gliomas ārstēšana
Lithuanian	Eflornitinas	Gliomos gydymas
Maltese	Eflornithine	Kura tal-glioma
Polish	Eflornityna	Leczenie glejaka
Portuguese	Eflornitina	Tratamento do glioma
Romanian	Eflornitină	Tratamentul gliomului
Slovak	Eflornitín	Liečba gliómu
Slovenian	Eflornitin	Zdravljenje glioma
Spanish	Eflornitina	Tratamiento del glioma
Swedish	Eflornitin	Behandling av gliom
Norwegian	Eflornitin	Behandling av gliom
Icelandic	Eflornithín	Meðferð á glíóma

<sup>1</sup> At the time of designation