



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Olaptesed pegol for the treatment of glioma

On 19 November 2014, orphan designation (EU/3/14/1364) was granted by the European Commission to Noxxon Pharma AG, Germany, for olaptesed pegol 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 (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 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 2.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 112,000 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 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 511,100,000 (Eurostat 2014).



The sponsor has provided sufficient information to show that olaptosed pegol might be of significant benefit for patients with glioma because studies in experimental models showed that the medicine might have improved effects compared with currently available 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?**

Olaptosed pegol works by blocking a protein called stromal cell derived factor-1 (SDF-1), which plays a role in the development of new blood vessels needed by the tumour to grow. By blocking SDF-1, the medicine is expected to prevent the tumour from being well supplied by new blood vessels, eventually killing the tumour cells.

SDF-1 also plays a role in causing the tumour cells to move (metastasise) to other parts of the body. By blocking the action of SDF-1, this medicine is also expected to reduce the spread of the tumour.

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

The effects of olaptosed have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with olaptosed in patients with glioma were planned.

At the time of submission, olaptosed was not authorised anywhere in the EU for glioma. Orphan designation of olaptosed had been granted in the United States for treatment of glioblastoma in conjunction with radiotherapy.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 October 2014 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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Olaptosed pegol	Treatment of glioma
Bulgarian	Олаптезед пегол	Лечение на глиома
Croatian	Olaptosedpegol	Liječenje glioma
Czech	Olaptosed pegol	Léčba gliomů
Danish	Olaptosed pegol	Behandling af gliom
Dutch	Olaptosed pegol	Behandeling van glioma
Estonian	Olapteseed-pegool	Glioomi ravi
Finnish	Olaptesedipegoli	Gliooman hoito
French	olaptésed pégol	Traitement des gliomes
German	Olaptosed Pegol	Behandlung von Gliomen
Greek	Ολαπτεσέδη πεγκόλη	Θεραπεία του γλοιώματος
Hungarian	Olapteszed	Glioma kezelése
Italian	Olaptosed pegol	Trattamento del glioma
Latvian	Olapteseda pegols	Gliomas ārstēšana
Lithuanian	Olaptesedas pegolas	Gliomos gydymas
Maltese	Olaptosed pegol	Kura tal-glioma
Polish	Olaptezed pegol	Leczenie glejaka
Portuguese	Olaptosed pegol	Tratamento do glioma
Romanian	Olaptosed pegol	Tratamentul gliomului
Slovak	Olaptezed pegol	Liečba gliómu
Slovenian	Olaptosed pegol	Zdravljenje glioma
Spanish	Olaptosed pegol	Tratamiento del glioma
Swedish	Olaptosed pegol	Behandling av gliom
Norwegian	Olaptosed pegol	Behandling av gliom
Icelandic	Ólap-unnið pegól	Meðferð á glíóma

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