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Public summary of opinion on orphan designation

Picropodophyllin for the treatment of glioma

On 23 August 2017, orphan designation (EU/3/17/1904) was granted by the European Commission to Axelar AB, Sweden, for picropodophyllin 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 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^{*}, 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.

^{*}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 515,700,000 (Eurostat 2017).

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with glioma because early results showed responses in patients whose disease had not responded to or had come back after other standard 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?

The way that picropodophyllin works is not completely understood, but it is thought to act in two ways: firstly, by blocking the action of a protein called IGF-1R (insulin-like growth factor-1 receptor), which is known to be present in excess in many cancers, including glioma, and which stimulates their growth; secondly, by interfering with a process called microtubule formation, which is needed for cells to be able to divide. The combination of these two actions is expected to reduce the growth of the glioma cells and help prevent the spread of the cancer.

What is the stage of development of this medicine?

The effects of picropodophyllin have been evaluated in experimental models.

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

At the time of submission, picropodophyllin 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 13 July 2017 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	Active ingredient	Indication
English	Picropodophyllin	Treatment of glioma
Bulgarian	Пикроподофилин	Лечение на глиома
Croatian	Pikropodofilin	Liječenje glioma
Czech	Pikropodofylin	Léčba gliomů
Danish	Picropodophyllin	Behandling af gliom
Dutch	Picropodofylline	Behandeling van glioma
Estonian	Pikropodofülliin	Glioomi ravi
Finnish	Pikropodofylliini	Gliooman hoito
French	Picropodophylline	Traitement des gliomes
German	Picropodophyllin	Behandlung von Gliomen
Greek	Πικροποδοφυλλίνη	Θεραπεία του γλοιώματος
Hungarian	Pikropodofilin	Glioma kezelése
Italian	Picropodofilina	Trattamento del glioma
Latvian	Pikropodofilīns	Gliomas ārstēšana
Lithuanian	Pikropodofilinas	Gliomos gydymas
Maltese	Pikropodofilin	Kura tal-glioma
Polish	Pikropodofilina	Leczenie glejaka
Portuguese	Picropodofilina	Tratamento do glioma
Romanian	Picropodofilină	Tratamentul gliomului
Slovak	Pikropodofilín	Liečba gliómu
Slovenian	Pikroprodofilin	Zdravljenje glioma
Spanish	Picropodofilina	Tratamiento del glioma
Swedish	Pikropodofyllin	Behandling av gliom
Norwegian	Pikropodofyllin	Behandling av gliom
Icelandic	Píkrópódófýllín	Meðferð á glíóma

¹ At the time of designation