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Public summary of opinion on orphan designation

Onfekafusp alfa for the treatment of glioma

On 26 June 2020, orphan designation EU/3/20/2291 was granted by the European Commission to Philogen S.p.A., Italy, for onfekafusp alfa for the treatment of glioma.

What is glioma?

Glioma is a brain tumour that affects the glial cells (the cells that surround and support the nerve cells). It mainly affects adults aged over 45 years, but it can occur at any age. Patients with glioma can have severe symptoms, the nature of which depends on where the tumour develops in the brain.

Symptoms can include headaches, nausea (feeling sick), loss of appetite, vomiting, muscle weakness in one part of the body 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 surgery, radiotherapy (treatment with radiation), and chemotherapy (medicines to treat cancer). Patients also received treatments for the symptoms of glioma, including corticosteroids to reduce pressure within the skull and medicines to prevent seizures.

*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, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).

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The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with glioma because early results in laboratory models suggest improvement when the medicine is added to existing 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 medicine is made of a protein called tumour necrosis factor (TNF) joined to an antibody (a type of protein). The antibody is designed to target new blood vessels (such as those in glioma tumours) where the TNF linked to it is expected to kill tumour cells and damage new blood vessels. Because new blood vessels supplying oxygen are important for tumours to growth, the medicine is expected to slow down the growth of the cancer and improve the symptoms of the disease.

What is the stage of development of this medicine?

The effects of the medicine 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, onfekafusp alfa was not authorised anywhere in the EU for the treatment of glioma or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 20 May 2020, 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

Contact details of the current sponsor for this orphan designation can be found on EMA website.

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[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Onfekafusp alfa	Treatment of glioma
Bulgarian	Онфекафусп алфа	Лечение на глиома
Croatian	Onfekafusp alfa	Liječenje glioma
Czech	Onfekafusp alfa	Léčba gliomů
Danish	Onfekafusp alfa	Behandling af gliom
Dutch	Onfekafusp alfa	Behandeling van glioma
Estonian	Alfa-onfekafusp	Glioomi ravi
Finnish	Onfekafuspi alfa	Glioman hoito
French	Onfekafusp alfa	Traitement des gliomes
German	Onfekafusp alfa	Behandlung von Gliomen
Greek	Onfekafusp alfa	Θεραπεία του γλοιώματος
Hungarian	Onfekafusp alfa	Glioma kezelése
Italian	Onfekafusp alfa	Trattamento del glioma
Latvian	Alfa-onfekafusps	Gliomas ārstēšana
Lithuanian	Onfekafuspas alfa	Gliomos gydymas
Maltese	Onfekafusp alfa	Kura tal-glioma
Polish	Onfekafusp alfa	Leczenie glejaka
Portuguese	Onfecafusp alfa	Tratamento do glioma
Romanian	Onfekafusp alfa	Tratamentul gliomului
Slovak	Onfekafusp alfa	Liečba gliómu
Slovenian	Onfekafusp alfa	Zdravljenje glioma
Spanish	Onfekafusp alfa	Tratamiento del glioma
Swedish	Onfekafusp alfa	Behandling av gliom
Norwegian	Onfekafusp alfa	Behandling av gliom
Icelandic	Onfekafusp alfa	Meðferð á glíóma

[1] At the time of designation