



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

10 May 2021
EMADOC-628903358-3723

Public summary of opinion on orphan designation

Human interleukin 12 fused with immunoglobulin G4 C-terminal Fc fragment for the treatment of glioma

On 9 December 2020, orphan designation EU/3/20/2368 was granted by the European Commission to VH Regulatory Consulting GmbH & Co. KG, Germany, for human interleukin 12 fused with immunoglobulin G4 C-terminal Fc fragment 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

*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).



(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.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with glioma because results from laboratory studies suggest that adding the medicine to temozolomide (chemotherapy) and radiotherapy leads to greater benefits.

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 contains interleukin-12 (IL-12), a protein that activates the immune system (the body's natural defences), and is expected to work by helping the immune system to fight the cancer. In this medicine, IL-12 is combined with a type of protein called immunoglobulin G4 fragment, which helps IL-12 stay active for longer. The medicine is expected to be delivered directly into the tumour.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of the medicine in experimental models was ongoing.

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

At the time of submission, the medicine 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 5 November 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.