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Public summary of opinion on orphan designation

Dabrafenib mesylate for the treatment of glioma

On 9 December 2020, orphan designation EU/3/20/2372 was granted by the European Commission to Novartis Europharm Limited, Ireland, for dabrafenib mesylate 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 studies suggest that some patients with advanced glioma whose cancer cells have a specific genetic mutation (change) called 'BRAF V600' had no sign of the cancer after receiving the medicine.

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?

Some patients with glioma have mutations in the gene for BRAF, a protein involved in stimulating cell division. This leads to the production of an abnormal form of BRAF which plays a role in the development of the cancer by allowing uncontrolled division of the tumour cells. Dabrafenib mesylate works by blocking the action of the abnormal BRAF, thereby helping to slow down the growth of the cancer.

What is the stage of development of this medicine?

The effects of dabrafenib mesylate have been evaluated in experimental models.

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

At the time of submission, dabrafenib mesylate was authorised in the EU, under the trade name Tafinlar, for the treatment of melanoma (a skin cancer) and non-small cell lung cancer.

At the time of submission, dabrafenib mesylate was not authorised anywhere in the EU for the treatment of glioma. Orphan designation of dabrafenib mesylate had been granted in the United States for the 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.