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# Public summary of opinion on orphan designation

Regorafenib for the treatment of glioma



On 28 June 2019, orphan designation EU/3/19/2177 was granted by the European Commission to Bayer AG, Germany, for regorafenib for the treatment of glioma.

## What is glioma?

Glioma is a tumour in the brain or spinal cord that affects the glial cells (cells that surround and support nerve cells). Patients with glioma can have severe symptoms, but the types of symptoms depend on where the tumour develops.

Symptoms can include headaches, nausea (feeling sick), loss of appetite, vomiting, weakness in the arms and legs, 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 (anti-inflammatory medicines) 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 518,400,000 (Eurostat 2019).

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The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with glioma. Early studies indicate that patients with glioma that has come back may live longer when treated with regorafenib than with an authorised treatment. 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?

Regorafenib is a 'protein kinase inhibitor'. This means that it blocks several enzymes that are important for the development of a blood supply to tumours and for the growth and development of cancer cells. By blocking the action of these enzymes, it is expected that regorafenib can stop the growth and spread of the glioma and so prevent symptoms from getting worse.

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

The effects of regorafenib have been evaluated in experimental models.

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

At the time of submission, regorafenib was authorised in the EU (as Stivarga) for colorectal cancer, gastrointestinal stromal tumour and hepatocellular cancer. It 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 23 May 2019, 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.

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 | Regorafenib | Treatment of glioma |
| Bulgarian | Регорафениб | Лечение на глиома |
| Croatian | Regorafenib | Liječenje glioma |
| Czech | Regorafenib | Léčba gliomů |
| Danish | Regorafenib | Behandling af gliom |
| Dutch | Regorafenib | Behandeling van glioma |
| Estonian | Regorafeniib | Glioomi ravi |
| Finnish | Regorafenibi | Gliooman hoito |
| French | Régorafénib | Traitement des gliomes |
| German | Regorafenib | Behandlung von Gliomen |
| Greek | Ρεγοραφενίμπη | Θεραπεία του γλοιώματος |
| Hungarian | Regorafenib | Glioma kezelése |
| Italian | Regorafenib | Trattamento del glioma |
| Latvian | Regorafenibs | Gliomas ārstēšana |
| Lithuanian | Regorafenibas | Gliomos gydymas |
| Maltese | Regorafenib | Kura tal-glioma |
| Polish | Regorafenib | Leczenie glejaka |
| Portuguese | Regorafenib | Tratamento do glioma |
| Romanian | Regorafenib | Tratamentul gliomului |
| Slovak | Regorafenib | Liečba gliómu |
| Slovenian | Regorafenib | Zdravljenje glioma |
| Spanish | Regorafenib | Tratamiento del glioma |
| Swedish | Regorafenib | Behandling av gliom |
| Norwegian | Regorafenib | Behandling av gliom |
| Icelandic | Regorafenib | Meðferð á glíóma |

[1] At the time of designation