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Public summary of opinion on orphan designation

Marizomib for the treatment of glioma

On 14 December 2018, orphan designation (EU/3/18/2119) was granted by the European Commission to Celgene Europe B.V., the Netherlands, for marizomib 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 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.

*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 517,400,000 (Eurostat 2018).

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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 showed that patients with an aggressive form of glioma called glioblastoma multiforme and whose disease progressed despite previous treatment had improvements with this medicine or their disease did not get worse. 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?

In glioma, overactive proteins called proteasomes prevent the cancer cells from dying normally and allow the cancer to grow. Marizomib is expected to block the activity of proteasomes, leading to death of the cancer cells.

What is the stage of development of this medicine?

The effects of marizomib 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, marizomib was not authorised anywhere in the EU for glioma. Orphan designation of the medicine had been granted in the United States for glioma.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 November 2018 recommending the granting of this designation.

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

[1] At the time of designation