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EMA/COMP/510448/2016
Committee for Orphan Medicinal Products

# Public summary of opinion on orphan designation

## Temozolomide for the treatment of glioma

On 29 August 2016, orphan designation (EU/3/16/1733) was granted by the European Commission to Double Bond Pharmaceutical AB, Sweden, for temozolomide 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 experienced depend on where the tumour develops in the brain.

Symptoms can include headaches, nausea, vomiting, loss of appetite 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 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 134,000 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, including temozolomide given by mouth) to improve survival. 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 513,700,000 (Eurostat 2016).

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The sponsor has provided sufficient information to show that this medicine (temozolomide applied as a gel in the brain) might be of significant benefit for patients with glioma because early studies showed that it may improve overall survival compared with currently authorised medicines. 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?

Temozolomide is a cancer medicine that has been authorised in the EU for several years to treat glioma. It belongs to a group of cancer medicines called alkylating agents. In the body, temozolomide is converted into another compound called MTIC. MTIC attaches itself to the DNA of cells while they are reproducing, which stops cell division. As a result, the tumour cells in the brain cannot reproduce, and this slows down the growth of the glioma.

This medicine will be available as a gel to be applied in the brain after surgical removal of the tumour.

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

The effects of temozolomide have been evaluated in experimental models.

At the time of submission of the application for orphan designation, a clinical trial with the medicine in patients with glioma had been completed and a further study was planned.

At the time of submission, this medicine was not authorised anywhere in the EU for glioma or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 July 2016 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 EMA website, on the medicine's rare disease designations page.

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 | Temozolomide | Treatment of glioma |
| Bulgarian | Темозоломид | Лечение на глиома |
| Croatian | Temozolomid | Liječenje glioma |
| Czech | Temozolomid | Léčba gliomů |
| Danish | Temozolomid | Behandling af gliom |
| Dutch | Temozolomide | Behandeling van glioma |
| Estonian | Temosolomiid | Glioomi ravi |
| Finnish | Temotsolomidi | Glioman hoito |
| French | Témozolomide | Traitement des gliomes |
| German | Temozolomid | Behandlung von Gliomen |
| Greek | Τεμοζολομίδη | Θεραπεία του γλοιώματος |
| Hungarian | Temozolomid | Glioma kezelése |
| Italian | Temozolomide | Trattamento del glioma |
| Latvian | Temozolomīds | Gliomas ārstēšana |
| Lithuanian | Temozolomidas | Gliomos gydymas |
| Maltese | Temozolomide | Kura tal-glioma |
| Polish | Temozolomid | Leczenie glejaka |
| Portuguese | Temozolomida | Tratamento do glioma |
| Romanian | Temozolomidă | Tratamentul gliomului |
| Slovak | Temozolomid | Liečba gliómu |
| Slovenian | Temozolomid | Zdravljenje glioma |
| Spanish | Temozolomida | Tratamiento del glioma |
| Swedish | Temozolomid | Behandling av gliom |
| Norwegian | Temozolomid | Behandling av gliom |
| Icelandic | Temózólómíð | Meðferð á glíóma |

[1] At the time of designation