22 September 2016
EMA/COMP/509421/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Zoledronic acid for the treatment of glioma

On 29 August 2016, orphan designation (EU/3/16/1735) was granted by the European Commission to Laboratorio Italiano Biochimico Farmaceutico Lisapharma S.p.A., Italy, for zoledronic acid 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 1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 51,000 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) 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 zoledronic acid might be of significant benefit for patients with glioma because laboratory studies indicate that it may lead to significant reductions in tumour growth, compared with authorised treatments. 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?

Zoledronic acid belongs to a group of medicines called 'bisphosphonates'. It has been authorised in the EU for several years to treat diseases affecting the bones such as osteoporosis.

In glioma, zoledronic acid is expected to work by blocking the action of an enzyme called farnesyl pyrophosphate synthase, which is involved in cell growth and survival. By blocking this enzyme, zoledronic acid is expected to slow down the growth of the glioma cells and therefore reduce the size of the tumour.

The zoledronic acid in this medicine is contained within tiny particles that transport zoledronic acid into the brain.

What is the stage of development of this medicine?

The effects of zoledronic acid have been evaluated in experimental models.

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

At the time of submission, zoledronic acid 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	Zoledronic acid	Treatment of glioma
Bulgarian	Золедронова киселина	Лечение на глиома
Croatian	Zoledronatna kiselina	Liječenje glioma
Czech	Kyselina zolendrová	Léčba gliomů
Danish	Zoledronsyre	Behandling af gliom
Dutch	Zoledroninezuur	Behandeling van glioma
Estonian	Zoledroonhape	Glioomi ravi
Finnish	Tsoledronihappo	Gliooman hoito
French	Acide zolédronique	Traitement des gliomes
German	Zoledronsäure	Behandlung von Gliomen
Greek	Ζολεδρονικό οξύ	Θεραπεία του γλοιώματος
Hungarian	Zoledronsav	Glioma kezelése
Italian	Acido zoledronico	Trattamento del glioma
Latvian	Zoledronskābe	Gliomas ārstēšana
Lithuanian	Zoledrono rūgštis	Gliomos gydymas
Maltese	Zoledronic acid	Kura tal-glioma
Polish	Kwas zoledronowy	Leczenie glejaka
Portuguese	Ácido zoledrónico	Tratamento do glioma
Romanian	Acid zoledronic	Tratamentul gliomului
Slovak	Kyselina zoledrónová	Liečba gliómu
Slovenian	Zoledronska kislina	Zdravljenje glioma
Spanish	Ácido zoledrónico	Tratamiento del glioma
Swedish	Zoledronsyra	Behandling av gliom
Norwegian	Zoledronsyre	Behandling av gliom
Icelandic	Zóledrónic sýra	Meðferð á glíóma

[1] At the time of designation