

21 January 2014
EMA/COMP/704856/2013
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Allogeneic and autologous haptenised and irradiated cells and cell lysates derived from glioma for the treatment of glioma

On 16 January 2014, orphan designation (EU/3/13/1211) was granted by the European Commission to ERC Belgium, Belgium, for allogeneic and autologous haptenised and irradiated cells and cell lysates derived from glioma 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 (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 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 less than 3 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 154,000 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

*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 512,200,000 (Eurostat 2013).

(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.

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with glioma because early studies showed a reduction of tumour size in patients whose disease got worse after treatment with existing methods. This assumption will need to be confirmed at the time of marketing authorisation.

How is this medicine expected to work?

The medicine works by activating the patient's immune system (the body's natural defences) so that it attacks and kills the cancer cells. The medicine is prepared from the patient's own cancer cells ('autologous cells') and cancer cells from other patients ('allogeneic cells'), which are then modified in the laboratory. When the modified cells are injected into the patient, it is expected that the allogeneic cancer cells will help the immune system recognise the patient's own cancer cells as 'foreign' and stimulate an immune response against them, helping to slow down or stop the progression of the disease.

What is the stage of development of this medicine?

The effects of the medicinal product have been evaluated in experimental models.

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

At the time of submission, the medicinal product was not authorised anywhere in the EU for glioma. Orphan designation of the medicinal product 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 6 November 2013 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:

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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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Allogeneic and autologous haptenised and irradiated cells and cell lysates derived from glioma	Treatment of glioma
Bulgarian	Алогенни и аутоложни хаптенизирани, и облъчени клетки и клетъчни лизати, получени от глиом.	Лечение на глиом
Czech	Alogenní a autologní haptenizované a ozářené buňky a buněčné lysáty získané z glioblastomu	Léčba gliomů
Croatian	Alogene i autologne haptenizirane i ozračene stanice i stanični lizati dobiveni iz glioma	Liječenje glioma
Danish	Allogene og autologe hapteniserede og bestrålede celler og cellelysater, fremstillet af glioblastomer	Behandling af gliom
Dutch	Allogene, autologe, gehapteniseerde en bestraalde cellen en cellysaten, afgeleid van glioblastomen	Behandeling van glioma
Estonian	Glioomist saadud allogeensed ja autoloogsed hapteniseeritud ja kiiritatud rakud ja rakulüsaadid	Glioomi ravi
Finnish	Glioblastoomasta saadut allogeeniset ja autologiset haptenoidut ja säteilytetyt solut ja solulysaatit	Gliooman hoito
French	Cellules allogéniques et autologues, irradiées et hapténisées, et lysats cellulaires dérivés de glioblastome	Traitement des gliomes
German	Aus Glioblastom gewonnene allogene und autologe haptenisierte und bestrahlte Zellen und Zelllysate	Behandlung von Gliomen
Greek	Αλλογενή, αυτόλογα και ακτινοβολημένα κύτταρα και κυτταρικά λύματα, που προκύπτουν από γλοιοβλάστωμα	Θεραπεία του γλοιώματος
Hungarian	Glioblasztómából nyert allogén és autológ haptenizált és besugárzott sejtek és sejtízátumok	Glioma kezelése
Italian	Cellule e lisati cellulari allogenici e autologhi, irradiati e aptenizzati, derivati da glioblastoma	Trattamento del glioma
Latvian	No gliomas iegūtas, apstarotas un ar haptēniem apstrādātas alogēnas un autologas šūnas un šūnu lizāti	Gliomas ārstēšana

¹ At the time of designation

Language	Active ingredient	Indication
Lithuanian	Alogeninės ir autologinės haptenuotos ir apšvitintos ląstelės ir ląstelių lizatai, gauti iš gliomos	Gliomos gydymas
Maltese	Ċelloli u lysates ċellulari alloġeneċi u awtologi, irradijati u aptenizzati, imnisslin minn glioblastoma	Kura tal-glioma
Polish	Allogeniczne i autologiczne, haptenuowane i napromieniowane komórki i lizaty komórkowe otrzymane z komórek glejaka	Leczenie glejaka
Portuguese	Células e lisatos celulares alogénicos e autólogos, irradiados e haptenuados, derivados de glioblastoma	Tratamento do glioma
Romanian	Celule și lizate celulare alogene și autologe haptenuate și iradiate, derivate din gliom	Tratamentul gliomului
Slovak	Alogénne a autológne haptenuované a ožiarené bunky a bunkové lyzáty pochádzajúce z gliómu	Liečba gliómu
Slovenian	Alogenske in avtologne haptenuirane ter obsevane celice in celični lizati, ki izvirajo iz glioblastoma	Zdravljenje glioma
Spanish	Células y lisados celulares alogénicos y autólogos haptenuados e irradiados, derivados de glioblastoma	Tratamiento del glioma
Swedish	Allogena och autologa haptenuerade och bestrålade celler och celllys, framställda från glioblastom	Behandling av gliom
Norwegian	Allogene og autologe haptenuerte og bestrålte celler og cellelysater, avledet fra glioblastom.	Behandling av gliom
Icelandic	Ósamgena og samgena haptenueraðar og geislaðar frumur og frumulýsat, afleidd af taugakímsæxli	Meðferð á glíóma