11 December 2012
EMA/COMP/657711/2012
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

IL-12-secreting dendritic cells, loaded with autologous tumour lysate for the treatment of glioma

On 8 November 2012, orphan designation (EU/3/12/1058) was granted by the European Commission to Activartis Biotech GmbH, Austria, for IL-12-secreting dendritic cells loaded with autologous tumour lysate 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 a 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 is 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

*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 27), Norway, Iceland and Liechtenstein. This represents a population of 506,300,000 (Eurostat 2011).

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(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 the medicine might be of significant benefit for patients with glioma because it works in a different way to existing treatments and early studies show that it might improve the long-term outcome for these patients. 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?

This medicine is expected to work by activating the patient's immune system (the body's natural defences) so that it attacks and kills the cancer cells.

To prepare this medicine, immune system cells called dendritic cells are taken from the patient. The dendritic cells are then loaded with a mixture of proteins from the patient's tumour and enabled to release a substance called interleukin-12 (IL-12). When the dendritic cells are given back to the patient, the proteins from the tumour are expected to be recognised as 'foreign' by the immune system, resulting in the stimulation of an immune response against the cancer cells in the body. The IL-12 produced by the dendritic cells is expected to enhance the immune system response against the cancer cells.

What is the stage of development of this medicine?

The effects of this medicine have been evaluated in experimental models.

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

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 5 October 2012 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 languages[1], Norwegian and Icelandic

Language	Active substance	Indication
English	IL-12-secreting dendritic cells, loaded with autologous tumour lysate	Treatment of glioma
Bulgarian	IL-12 секретиращи дендритни клетки, заредени с автоложен туморен лизат	Лечение на глиома
Czech	IL-12 sekretující dendritické buňky, naložené autologním nádorovým lyzátem	Léčba gliomů
Danish	IL-12 secernerende dentritiske celler mættet med autologtumor lysater	Behandling af gliom
Dutch	IL-12- secreterende dendritische cellen, geladen met autoloog tumorlysaat	Behandeling van glioma
Estonian	IL-12 eritavad autoloogse tuumori lüsaadiga laetud dendriitrakud	Glioomi ravi
Finnish	IL-12:ta erittävät, autologisella tuumorilysaatilla ladatu dendriittisolut	Glioman hoito
French	Cellules dendritiques productrices d'IL-12, chargées avec un lysat tumoral autologue	Traitement des gliomes
German	Il-12 sezernierende dentritische Zellen, die mit autologem Tumormaterial beladen sind	Behandlung von Gliomen
Greek	Δενδριτικά κύτταρα που παράγουν IL-12 επωασμένα με κυτταρόλυμα αυτόλογου όγκου	Θεραπεία του γλοιώματος
Hungarian	IL-12-t szekretáló dendritikus sejtek autológ tumor lizátummal dúsítva	Glioma kezelése
Italian	Cellule dentritiche secernenti IL-12, pulsate con lisato tumorale autologo	Trattamento del glioma
Latvian	Il-12 izdalošas dendrītiskas šūnas, kurās ievadīts autologa audzēja lizāts	Gliomas ārstēšana
Lithuanian	IL-12 sekretuojančios dendritinės ląstelės, pripildytos autologinio tumoro lizatu	Gliomos gydymas
Maltese	Ċelloli dentritiċi li jipproduċu IL-12, mgħobbija b'lysate tumurali awtologuż	Kura tal-glioma
Polish	Komórki dendrytyczne wydzielające IL-12 z lizatem uzyskanym z autologicznego guza.	Leczenie glejaka
Portuguese	Células dendríticas produtoras de IL-12, carregadas com um lisado tumoral autólogo	Tratamento do glioma
Romanian	Celule dendritice secretoare de IL-12, încărcate cu lizat tumoral autolog	Tratamentul gliomului
Slovak	Dendriticke bunky vylučujúce IL-12, plnené autológnym nádorovým lyzátom	Liečba gliómu
Slovenian	Dentritične celice, ki izločajo IL-12, napolnjene z lizatom avtolognega tumorja	Zdravljenje glioma
Spanish	Celulas dentriticas secretoras de Interleukina-12 cargadas con el lisado de un tumor autologo	Tratamiento del glioma

[1] At the time of designation

Language	Active substance	Indication
Swedish	IL-12 utsöndrande dendritiska celler fyllda med autologt tumörlysat	Behandling av gliom
Norwegian	Dendrittiske celler som utskiller IL-12 og lastet med autologt tumor lysat	Behandling av gliom
Icelandic	IL-12 dendritika fruma, að ferma autolog æxli efni	Meðferð á glíóma