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EMA/COMP/228647/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Autologous dendritic cells pulsed with RNA from glioma stem cells for the treatment of glioma

On 4 June 2014, orphan designation (EU/03/14/1273) was granted by the European Commission to Epitarget AS, Norway, for autologous dendritic cells pulsed with RNA from glioma stem cells 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 2.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 112,000 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 511,100,000 (Eurostat 2014).

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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 this medicine might be of significant benefit for patients with glioma because early clinical studies showed that the medicine increased progression-free survival (how long patients lived without their disease getting worse) of patients when given in combination with currently 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?

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.

The medicine is prepared from the patient's own (autologous) immune cells called dendritic cells. These cells are mixed in the laboratory with genetic material from stem cells from the patient's glioma (cells from which the glioma is believed to originate and propagate). When the dendritic cells are injected back into the patient, it is expected that they will help the immune system recognise the patient's glioma 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?

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, the 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 9 April 2014 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:

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0349 Oslo
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E-mail: mail@epitarget.com

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	Autologous dendritic cells pulsed with RNA from glioma stem cells	Treatment of glioma
Bulgarian	Автоложни дендритни клетки, натоварени с РНК от глиомни стволови клетки	Лечение на глиома
Croatian	Autologne dendritičke stanice podražene s RNK iz matičnih stanica glioma	Liječenje glioma
Czech	Autologní dendritické buňky pulzované RNA z kmenových buněk gliomu	Léčba gliomů
Danish	Autologe dendritiske celler pulset med RNA fra gliom stamceller	Behandling af gliom
Dutch	Autologe dendritische cellen gepulseerd met RNA afkomstig van glioma stamcellen	Behandeling van glioma
Estonian	Glioomi tüvirakkude RNA-ga laetud autoloogsed dendriitrakud	Glioomi ravi
Finnish	Glioman kantasolujen RNA:lla ladatut autologiset dendriittisolut	Glioman hoito
French	Cellules dendritiques autologues chargées en ARN des cellules souches gliomales	Traitement des gliomes
German	Autologe dendritische Zellen, die mit Gliomastammzellen-RNS behandelt wurden	Behandlung von Gliomen
Greek	Αυτόλογα δενδριτικά κύτταρα επωασμένα με RNA από βλαστοκύτταρα γλοιώματος	Θεραπεία του γλοιώματος
Hungarian	Glióma őssejtekből származó RNS-sel pulzált autológ dendritikus sejtek	Glioma kezelése
Italian	Cellule dendritiche autologhe pulsate con RNA di cellule staminali di glioma	Trattamento del glioma
Latvian	Ar RNS no gliomas cilmes šūnām pārslogotas autologās dendrītu šūnas	Gliomas ārstēšana
Lithuanian	Autologinės dendritinės ląstelės pripildytos gliomos kamieninių ląstelių RNR	Gliomos gydymas
Maltese	Ċelluli dendritiċi awtologużi pulsati ma' RNA minn ċelluli staminali tal-glioma	Kura tal-glioma
Polish	Autologiczne komórki dendrytowe obładowane RNA z komórek macierzystych glejaka	Leczenie glejaka
Portuguese	Células dendríticas autólogas carregadas com ARN de células estaminais de glioma	Tratamento do glioma
Romanian	Celule dentriceautologe încărcate cu ARN extras din celule stem de gliom	Tratamentul gliomului
Slovak	Autológne dendritické bunky pulzované RNA z kmeňových buniek gliómu	Liečba gliómu

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Avtologne dendritične celice pulzirane z RNK matičnih gliomskih celic	Zdravljenje glioma
Spanish	Células dendríticas autólogas impulsadas con ARN de las células madre del glioma	Tratamiento del glioma
Swedish	Autologa dendritiska celler pulserade med RNA från gliomstamceller	Behandling av gliom
Norwegian	Autologe dendrittiske celler pulset med RNA fra stamceller fra gliomer	Behandling av gliom
Icelandic	Samgena griplufrumur hlaðnar RNA úr glíóma stofnfrumum	Meðferð á glíóma