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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Autologous dendritic cells pulsed with autologous tumour cell lysate for the treatment of glioma

On 15 February 2007, orphan designation (EU/3/07/431) was granted by the European Commission to Dorian Regulatory Affairs BV, The Netherlands, for autologous dendritic cells pulsed with autologous tumour cell lysate for the treatment of glioma.

The sponsorship was transferred to Northwest Biotherapeutics GmbH, Germany, in May 2012.

What is glioma?

Tumours that arise in the brain tissue are known as primary brain tumours. Primary brain tumours are classified by the type of tissue from which they originate, the most common being gliomas, which arise in the glial (supportive) tissue. Patients affected by gliomas can suffer from severe symptoms of the nervous system, depending on where in the brain the tumour develops. Gliomas are life-threatening.

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 46,000 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?

Treatment for glioma depends on a number of factors and encompasses several methods such as surgery, radiotherapy (using high-dose x-rays or other high-energy rays to kill cancer cells) or chemotherapy (using drugs to kill cancer cells), as well as some symptomatic treatments. Symptomatic treatments include certain steroid hormones (corticosteroids) to control the effects of raised pressure within the skull, and medication to help control seizures, as required. Several products

*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.
At the time of designation, this represented a population of 500,300,000 (Eurostat 2007).

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for the treatment of glioma were authorised in the Community at the time of submission of the application for orphan designation.

Autologous dendritic cells pulsed with autologous tumour cell lysate might be of potential significant benefit for the treatment of glioma because it might improve the long-term outcome of the patients. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

Autologous dendritic cells pulsed with autologous tumour cell lysate consists of cancer cells of the patient. Once extracted from the patient the cancer cells are modified to increase their ability to create an immune response. The cells are then killed and given to the patient with the aim of activating the patient's immune system against any cancer cells, which could remain in the body after surgery, thus reducing the possibility of a relapse.

What is the stage of development of this medicine?

The effects of autologous dendritic cells pulsed with autologous tumour cell lysate were evaluated in experimental models.

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

Autologous dendritic cells pulsed with autologous tumour cell lysate was not authorised anywhere worldwide for treatment of glioma or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 10 January 2007 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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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 autologous tumour cell lysate	Treatment of glioma
Bulgarian	Автоложни дендритни клетки, натоварени с автоложен туморно клетъчен лизат	Лечение на глиома
Czech	Autologní dendritické buňky s lyzátem autologních mozkových nádorových buněk	Léčba gliomů
Danish	Autologe dendritiske celler pulset med autologt lysat af hjernetumorceller	Behandling af gliom
Dutch	Autologe dendritische cellen gepulseerd met autoloog tumorcellysaat	Behandeling van glioma
Estonian	Autoloogse ajukasvaja rakulüsaadiga laetud autoloogsed dendriitrakud	Glioomi ravi
Finnish	Autologisella aivokasvainsolulysaatilla ladatut autologiset dendriittisolut	Glioman hoito
French	Cellules dendritiques autologues chargées en lysat cellulaire tumoral autologue	Traitement des gliomes
German	Autologe endrit mit autologem umorelllysat	Behandlung von Gliomen
Greek	Αυτόλογα δενδριτικά κύτταρα παλμικά επιφορτισμένα με κυτταρόλυμα αυτόλογου όγκου	Θεραπεία του γλοιώματος
Hungarian	Autológ tumor sejtlizátum hordozót tartalmazó autológ dendritikus sejtek	Glioma kezelése
Italian	Cellule dendritiche autologhe pulsate con lisato di cellule tumorali autologhe	Trattamento delglioma
Latvian	Ar autologu smadzeņu audzēju šūnu lizātu pārslogotas autologās dendrītu šūnas	Gliomas ārstēšana
Lithuanian	Autologinės dendritinės ląstelės su smegenų auglio autologinių ląstelių lizatu	Gliomos gydymas
Maltese	Ċelluli dendritiċi awtologużi pulsati ma' lysate ta' ċelluli tumurali awtologużi	Kura tal-glioma
Polish	Autologiczne komórki dendrytowe obładowane lizatem autologicznych komórek guza mózgu	Leczenie glejaka
Portuguese	Células dendríticas autólogas carregadas com lisado de células tumorais autólogas	Tratamento do glioma
Romanian	Celule dendritice de tip autolog încărcate cu lizat celular de tip autolog extras dintr-o tumoră cerebrală	Tratamentul gliomului
Slovak	Autológové dendritové bunky naplnené lyzátom autológových buniek tumoru mozgu	Liečba gliómu
Slovenian	Avtologne dendritične celice, naložene z lizatom avtlognih celic možganskega tumorja	Zdravljenje glioma

[1] At the time of transfer of sponsorship

Language	Active ingredient	Indication
Spanish	Células dendríticas autólogas cargadas de lisado autólogo de células de tumor	Tratamiento del glioma
Swedish	Autologa dendritiska celler pulserade med autologt tumörscellysat	Behandling av gliom
Norwegian	Autologe dendritiske celler pulset med autologt tumorcellelysat	Behandling av gliom
Icelandic	Samgena angafrumur hlaðnar uppleystu frumuleysiúr samgena heilaæxli	Meðhöndlun á glíóma