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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Autologous dendritic cells pulsed with tumour antigen-derived synthetic peptides (MAGE-1, HER-2, AIM-2, TRP-2, gp-100, and interleukin-13 receptor alpha) for the treatment of glioma

On 19 February 2014, orphan designation (EU/3/14/1247) was granted by the European Commission to Diamond BioPharm Limited, United Kingdom, for autologous dendritic cells pulsed with tumour antigen-derived synthetic peptides (MAGE-1, HER-2, AIM-2, TRP-2, gp-100, and interleukin-13 receptor alpha) 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 not more than 2 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 102,000 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).

*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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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.

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 survival of patients who were newly diagnosed with glioma. 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?

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 certain patient's own (autologous) immune cells, which are called dendritic cells. These cells are mixed in the laboratory with fragments of certain proteins called MAGE-1, HER-2, AIM-2, TRP-2, gp-100 and interleukin13-receptor, which are found in large amounts on the surface of certain glioma cells. When the dendritic cells are injected back into the patient, it is expected that they 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 medicine have been evaluated in experimental models.

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 January 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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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 tumour antigen-derived synthetic peptides (MAGE-1, HER-2, AIM-2, TRP-2, gp-100, and interleukin-13 receptor alpha)	Treatment of glioma
Bulgarian	Автоложни дендритни клетки, пулсирани със синтетични пептиди, извлечени от туморен антиген (MAGE-1, HER-2, AIM-2, TRP-2, gp100 и интерлевкин-13 рецептор алфа)	Лечение на глиома
Czech	Autologní dendritické buňky pulzované syntetickými peptidy odvozenými z nádorových antigenů (MAGE-1, HER-2, AIM-2, TRP-2, gp100 a alfa-receptoru pro interleukin-13)	Léčba gliomů
Croatian	Autologne dendritičke stanice podražene sintetičkim peptidima dobivenim od tumorskih antigena (MAGE-1, HER-2, AIM-2, TRP-2, gp100 i interleukin-13 receptor alfa)	Liječenje glioma
Danish	Autologe dendritiske celler pulseret med tumor-antigen-afledte syntetiske peptider (MAGE-1, HER-2, AIM-2, TRP-2, gp100 og interleukin-13-receptor-alfa)	Behandling af gliom
Dutch	Autologe dendritische cellen gepulseerd met synthetische peptiden afgeleid van tumorantigenen (MAGE-1, HER-2, AIM-2, TRP-2, gp100 en interleukine-13-receptor alfa)	Behandeling van glioma
Estonian	Autoloogsed dendriitrakud, mida on pulseerivalt töödeldud tuumori antigeenist tuletatud sünteetiliste peptiididega (MAGE-1, HER-2, AIM-2, TRP-2, gp100 ja interleukiin-13 retseptor alfa)	Glioomi ravi
Finnish	Autologiset dendriitisolut, jotka on pulssitettu kasvaimen antigeeni-johdettujen synteettisten peptidien (MAGE-1, HER-2, AIM-2, TRP-2, gp100 ja interleukiini-13:n reseptori alfa) kanssa	Glioman hoito
French	Cellules dendritiques autologues pulsées par des peptides synthétiques (MAGE-1, HER-2, AIM-2, TRP-2, gp100 et récepteur alpha de l'interleukine 13) dérivés d'un antigène tumoral	Traitement des gliomes
German	Autologe dendritische Zellen, die mit synthetischen Peptiden aus Tumorantigenen (MAGE-1, HER-2, AIM-2, TRP-2, gp100 und Interleukin-13-Rezeptor alpha) beladen („gepulst“) wurden	Behandlung von Gliomen

[1] At the time of designation

Language	Active ingredient	Indication
Greek	Αυτόλογα δενδριτικά κύτταρα επωασμένα με συνθετικά πεπτίδια προερχόμενα από αντιγόνα όγκων (MAGE-1, HER-2, AIM-2, TRP-2, gp100 και α-αλυσίδα του υποδοχέα της ιντερλευκίνης-13)	Θεραπεία του γλοιώματος
Hungarian	Tumor antigénből származó szintetikus peptidekkel (MAGE-1, HER-2, AIM-2, TRP-2, gp100, és interleukin-13 receptor alfa) transzformált autológ dendritsejtek	Glioma kezelése
Italian	Cellule dendritiche autologhe pulsate con peptidi sintetici derivati dagli antigeni tumorali (MAGE-1, HER-2, AIM-2, TRP-2, gp100 e recettore alfa per l'interleuchina-13)	Trattamento del glioma
Latvian	Ar no audzēju antigēniem atvasinātiem sintētiskiem peptīd (MAGE-1, HER-2, AIM-2, TRP-2, gp-100 un interleikīna-13 alfa receptoru) pārslogotas autologas dendrītiskās šūnas	Gliomas ārstēšana
Lithuanian	Autologinės dendritinės ląstelės, pripildytos naviko antigeno išskirtais sintetiniais baltymais (MAGE-1, HER-2, AIM-2, TRP-2, gp100 ir interleukino-13 alfa receptorius)	Gliomos gydymas
Maltese	Ċelloli dendritiċi awtologużi pulsati b'peptidi sintetiċi imnissla minn antiġen ta' tumur (MAGE-1, HER-2, AIM-2, TRP-2, gp100 u riċettur alpha għall-interleukin-13)	Kura tal-glioma
Polish	Autologiczne komórki dendrytyczne z wprowadzonymi syntetycznymi peptydami antygenów nowotworowych (MAGE-1, HER-2, AIM-2, TRP-2, gp100 oraz receptor alfa interleukiny 13)	Leczenie glejaka
Portuguese	Células dendríticas autólogas estimuladas com peptídeos sintéticos antigénicos derivados do tumor (MAGE-1, HER-2, AIM-2, TRP-2, gp100 e interleucina 13 do recetor-alfa)	Tratamento do glioma
Romanian	Celule dendritice autologe stimulate cu peptide sintetice derivate din antigene tumorale (MAGE-1, HER-2, AIM-2, TRP-2, gp100 şi receptorul alfa al interleukinei-13)	Tratamentul gliomului
Slovak	Autológne dendritické bunky pulzované syntetickými peptidmi derivovanými z nádorových antigénov (MAGE-1, HER-2, AIM-2, TRP-2, gp100 a alfa receptora pre interleukín-13)	Liečba gliómu
Slovenian	Avtologne dendritične celice, pulzirane s sintetičnimi peptidi, pridobljenimi iz tumorskih antigenov (MAGE-1, HER-2, AIM-2, TRP-2, gp100 in receptorja alfa za interlevkin-13)	Zdravljenje glioma

Language	Active ingredient	Indication
Spanish	Células dendríticas autólogas pulsadas con péptidos sintéticos derivados de antígenos tumorales (MAGE-1, HER-2, AIM-2, TRP-2, gp100 y receptor alfa de interleucina 13)	Tratamiento del glioma
Swedish	Autologa dendritiska celler pulsade med syntetiska peptider härledda från tumörantigen (MAGE-1, HER-2, AIM-2, TRP-2, gp100 och interleukin-13-receptor alfa)	Behandling av gliom
Norwegian	Autologe dendrittiske celler pulsert med tumorantigenavledete syntetiske peptider (MAGE-1, HER-2, AIM-2, TRP-2, gp100 og interleukin-13-reseptor-alfa)	Behandling av gliom
Icelandic	Samgena griplu frumur púlseraðar með æxlis mótefnavaka samtengdum peptíðum (MAGE-1, HER-2, AIM-2, TRP-2, gp100, og interleukín-13-viðtaka alfa)	Meðferð á glíóma