27 August 2014
EMA/COMP/363867/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Humanised recombinant monoclonal antibody against epidermal growth factor receptor conjugated to maleimidocaproyl monomethylauristatin F for the treatment of glioma

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On 29 July 2014, orphan designation (EU/3/14/1305) was granted by the European Commission to AbbVie Ltd, the United Kingdom, for humanised recombinant monoclonal antibody against epidermal growth factor receptor conjugated to maleimidocaproyl monomethylauristatin F 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 2 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer 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 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 studies suggest it could reduce tumour size in patients resistant to other treatments, when the medicine is used in combination with standard treatment. 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 made up of a monoclonal antibody (a type of protein) that has been designed to attach mainly to receptors on the surface of glioma cells called epidermal growth factor receptors (EGFRs). In glioma cells, these receptors are involved in the growth and spread of the tumour. The monoclonal antibody in this medicine is attached to monomethyl auristatin F, a cytotoxic (cell-killing) molecule, and is expected to deliver the monomethyl auristatin F to the glioma cells. Once inside the tumour cells, it is expected to kill them and thereby slow 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. Orphan designation has been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 June 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.

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For more information

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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.

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Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Humanised recombinant monoclonal antibody against epidermal growth factor receptor conjugated to maleimidocaproyl monomethylauristatin F	Treatment of glioma
Bulgarian	Хуманизирано рекомбинантно моноклонално антитяло срещу рецептора на епидермалния растежен фактор конюгирано с малеимидокапроил монометилауристатин F	Лечение на глиома
Croatian	Humanizirano rekombinantno monoklonsko protutijelo protiv receptora epidermalnog faktora rasta konjugirano s maleimidokaproilmonometilauristatinom F	Liječenje glioma
Czech	Humanizovaná rekombinantní monoklonální protilátka proti epidermálnímu receptoru růstového faktoru vázaná na maleimidokaproyl monomethlyauristatin F	Léčba gliomů
Danish	Humaniseret rekombinant monoklonalt antistof mod epidermal vækstfaktorreceptor konjugeret til maleimidocaproyl monomethylauristatin F	Behandling af gliom
Dutch	Gehumaniseerd recombinant monoclonaal antilichaam gericht tegen epidermale groeifactor-receptor geconjugeerd aan maleïmidocaproyl monomethylauristatin F	Behandeling van glioma
Estonian	Maleimidokaproüülmonometüülauristatiin F-ga konjugeeritud humaniseeritud rekombinantne monoklonaalne epidermaalse kasvufaktori retseptori vastane antikeha	Glioomi ravi
Finnish	Humanisoitu maleimidokaproyyli-monometyyliauristatiini F:ään konjugoitunut rekombinantti monoklonaalinen vasta-aine, joka estää epidermaalista kasvutekijäreseptoria	Glioman hoito
French	Anticorps monoclonal recombinant humanisé dirigé contre le récepteur du facteur de croissance épidermique conjugué à la maléimidocaproyle monométhylauristatine F	Traitement des gliomes
German	Humanisierter rekombinanter monoklonaler, mit Maleimidocaproyl-Monomethylauristan F konjugierter Antikörper gegen den Rezeptor für den Epidermalen Wachstumsfaktor	Behandlung von Gliomen

[1] At the time of designation

Language	Active ingredient	Indication
Greek	Ανθρωποποιημένο ανασυνδυασμένο μονοκλωνικό αντίσωμα έναντι του υποδοχέα του επιδερμικού αυξητικού παράγοντα συζευγμένο με μαλεϊμιδοκαπροϋλ μονομεθυλοριστατίνη F	Θεραπεία του γλοιώματος
Hungarian	Maleimidokaproil monometilaurisztatin F-konjugált epidermális növekedési faktor receptor-ellenes humanizált, rekombináns monoklonális antitest	Glioma kezelése
Italian	Anticorpo monoclonale umanizzato ricombinante contro il recettore del fattore di crescita dell'epidermide coniugato a Maleimidocaproil Monometilauristatina F	Trattamento del glioma
Latvian	Humanizēta rekombinanta monoklonāla antiviela pret epidermas augšanas faktora receptoru, kas konjugēta ar maleimidokaproila monometilauristatīnu F	Gliomas ārstēšana
Lithuanian	Humanizuotas rekombinantinis monokloninis antikūnas prieš epidermio augimo faktoriaus receptorius, sujungtas su maleimidokaproilo monometilauristatinu F	Gliomos gydymas
Maltese	Antikorp monoklonali umanizzat rikombinanti kontra r-riċettur tal-fattur għall-iżvilupp epidermali kkonjugat ma' maleimidocaproyl monomethylauristatin F	Kura tal-glioma
Polish	Humanizowane rekombinowane przeciwciało monoklonalne przeciw receptorowi nabłonkowego czynnika wzrostu sprzężone z maleimidokaproilomonometyloaurystatyną F	Leczenie glejaka
Portuguese	Anticorpo monoclonal humanizado recombinante contra o recetor do fator de crescimento epidérmico conjugado com maleimidocaproil monometilauristatina F	Tratamento do glioma
Romanian	Anticorp monoclonal recombinant umanizat anti receptor al factorului de creştere epidermal conjugat cu maleimidocaproil monometilauristatina F	Tratamentul gliomului
Slovak	Humanizovaná rekombinantná monoklonálna protilátka zacielená na receptor epidermálneho rastového faktora naviazaná na maleimidokaproyl monometylauristatín F	Liečba gliómu
Slovenian	Humanizirano rekombinantno monoklonsko protitelo proti humanemu receptorju za epidermalni rastni dejavnik, konjugiran na maleimidokaproil monometilauristatin F	Zdravljenje glioma
Spanish	Anticuerpo monoclonal recombinante humanizado contra el receptor del factor de crecimiento epidérmico conjugado con maleimidocaproil monometilauristatina F	Tratamiento del glioma

Language	Active ingredient	Indication
Swedish	Humaniserad rekombinant monoklonal antikropp mot epidermal tillväxtfaktorreceptor konjugerad med maleimidokaproyl monometylauristatin F	Behandling av gliom
Norwegian	Humanisert rekombinant monoklonalt antistoff mot epidermal vekstfaktorreseptor konjugert til maleimidokaproyl monometylauristatin F	Behandling av gliom
Icelandic	Mannaaðlagað raðbrigða einstofna mótefni gegn vaxtarþáttarviðtaka þekjufrumna (epidermal growth factor receptor) tengt maleímídócaprópýl mónómethýlauristatín	Meðferð á glíóma

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