EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

23 September 2013
EMA/COMP/526571/2012 Rev.2
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Humanised monoclonal antibody against epidermal growth factor receptor for the treatment of glioma

First publication	25 September 2012
Rev.1: transfer of sponsorship	11 March 2013
Rev.2: withdrawal from the Community Register	23 September 2013

Disclaimer

Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in July 2013 on request of the Sponsor.

On 9 August 2012, orphan designation (EU/3/12/1029) was granted by the European Commission to Abbott Laboratories, United Kingdom, for humanised monoclonal antibody against epidermal growth factor receptor for the treatment of glioma.

The sponsorship was transferred to AbbVie Ltd, United Kingdom, in January 2013.

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.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

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 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 (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 on the basis of studies in experimental models showing that the medicine in combination with temozolomide (an anticancer medicine authorized in the EU for the treatment of glioma) and radiotherapy might block tumour growth better than temozolomide and radiotherapy alone. 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 is a monoclonal antibody, a type of protein designed to recognise and target a specific structure found in certain cells. The medicine targets epidermal growth factor receptors (EGFR) found on the surface of some tumour cells. These receptors play an important role in the growth, progression and spread of the cancer. By attaching to and blocking their action, the medicine is expected to help slow down the growth of the cancer.

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 solid tumours 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. Orphan designation of the medicine had been granted in the United States of America for glioblastoma multiforme, a type of glioma.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 11 July 2012 recommending the granting of this designation.

[*]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 Lichtenstein.
At the time of designation, this represented a population of 509,000,000 (Eurostat 2012).

_____

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:

AbbVie Ltd
Abbott House
Vanwall Business Park
Vanwall Road
Maidenhead
Berks SL6 4XE
United Kingdom
Telephone: +44 1628 644501
Telefax: +44 1628 672624
E-mail: orphandrugs.enquiries@abbvie.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	Humanised monoclonal antibody against epidermal growth factor receptor	Treatment of glioma
Bulgarian	Хуманизирано моноклонално антитяло - срещу рецептора на епидермалния растежен фактор	Лечение на глиома
Czech	Humanizovaná monoklonální protilátka proti EGFR	Léčba gliomů
Danish	Humaniseret monoklonalt antistof mod epidermal vækstfaktor receptor	Behandling af gliom
Dutch	Gehumaniseerd monoklonaal antilichaam tegen epidermaal groeifactorreceptor	Behandeling van glioma
Estonian	Epidermaalse kasvufaktori retseptorii vastane humaniseeritud monoklonaalne antikeha	Glioomi ravi
Finnish	Humanisoitu monoklonaalinen anti-EGFR-vasta-aine	Glioman hoito
French	Anticorps monoclonal humanisé anti-EGFR	Traitement des gliomes
German	Humanisierter monoklonaler Antikörper gegen den Rezeptor für Epidermalen Wachstumsfaktor (EGF-R)	Behandlung von Gliomen
Greek	Εξανθρωποποιημένο Μονοκλωνικό Αντίσωμα έναντι του υποδοχέα του επιδερμιδικού αυξητικού παράγοντα	Θεραπεία του γλοιώματος
Hungarian	Humanizált monoklonális EGFR elleni antitest	Glioma kezelése
Italian	Anticorpo monoclonale umanizzato anti EGFR	Trattamento del glioma
Latvian	Humanizēta monoklonālā antiviela pret epidermālo augšanas faktoru receptoru (epidermal growth factor receptor - EGFR)	Gliomas ārstēšana
Lithuanian	Žmogaus monokloninis antikūnas prieš epidermio augimo faktoriaus receptorių	Gliomos gydymas
Maltese	Antikorp monoklonali uman kontra riċettur tal-fattur għall-iżvilupp epidermali	Kura tal-glioma
Polish	Humanizowane przeciwciało monoklonalne przeciw receptorowi czynnika wzrostu naskórka	Leczenie glejaka
Portuguese	Anticorpo monoclonal humanisado anti receptor do factor de crescimento da epiderme	Tratamento do glioma
Romanian	Anticorp monoclonal umanizat anti receptor pentru factorul de creştere epidermic	Tratamentul gliomului
Slovak	Humanizovaná monoklonálna protilátka proti receptoru epidermálneho rastového faktora	Liečba gliómu
Slovenian	Humanizirano monoklonsko protitelo proti EGFR	Zdravljenje glioma
Spanish	Anticuerpo monoclonal humanizado contra el receptor del factor de crecimiento epidérmico	Tratamiento del glioma

[1] At the time of designation

Language	Active ingredient	Indication
Swedish	Humaniserad monoklonal antikropp mot epidermal tillväxtfaktorreceptor	Behandling av gliom
Norwegian	Humanisert monoklonalt antistoff mot epidermal vekstfaktor-reseptor	Behandling av gliom
Icelandic	Mannaaðlagað einstofna mótefni gegn epidermal vaxtarþáttar viðtaka	Meðferð á glíóma