22 November 2018
EMA/514590/2018

Public summary of opinion on orphan designation

Autologous glioma tumour cells treated with antisense molecule directed against the insulin-like growth factor type 1 receptor for the treatment of glioma

On 24 August 2018, orphan designation (EU/3/18/2061) was granted by the European Commission to Pharma Gateway AB, Sweden, for autologous glioma tumour cells treated with antisense molecule directed against the insulin-like growth factor type 1 receptor for the treatment of glioma.

What is glioma?

Glioma is a 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 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 it 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 2.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 135,000 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 517,400,000 (Eurostat 2018).

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(medicines to treat cancer). Patients also received treatments for the symptoms of glioma, including corticosteroids to reduce pressure inside 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 with glioma because early studies have found that patients with glioblastoma (a fast-growing form of glioma) given the medicine before standard treatment lived longer and their condition remained stable for longer compared with standard treatment 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 consists of glioma cells taken from the patient's body, which are modified by treatment with an 'antisense molecule'. When the modified cells are injected back into the patient, they encourage the immune system (the body's defences) to regard glioma cells as foreign and to attack them. The antisense molecule also prevents the action of certain cells called tumour-associated macrophages which support and protect glioma cells. This makes the glioma cells more vulnerable to the immune system. By working in these different ways, the medicine is expected to stop the growth of gliomas.

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 glioblastoma were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for glioma. Orphan designation of the medicine had been granted in the United States for malignant glioma.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 19 July 2018 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

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 glioma tumour cells treated with antisense molecule directed against the insulin-like growth factor type 1 receptor	Treatment of glioma
Bulgarian	Автоложни туморни клетки от глиома, обработени с антисенс молекули, насочени срещу инсулиноподобния растежен фактор 1	Лечение на глиома
Croatian	Autologne stanice tumora glioma tretirane protusmjernom molekulom usmjerenom na receptor tipa 1 čimbenika rasta sličnog inzulinu	Liječenje glioma
Czech	Autologní nádorové gliové buňky ošetřené protisměrnou molekulou cílenou k receptoru insulin-like růstového faktoru	Léčba gliomů
Danish	Autolog glioma tumor celler behandlet med antisense molekyle rettet mod insulinlignende vækstfaktor type 1 receptor	Behandling af gliom
Dutch	Autologe gliomatumorcellen behandeld met antisense molecule gericht tegen “insuline-like” groeifactor type 1 receptor	Behandeling van glioma
Estonian	Autoloogsed glioomi kasvajarakud, mida on töödeldud insuliinisarnase kasvufaktori esimest tüüpi retseptori vastu suunatud *antisense* molekulidega	Glioomi ravi
Finnish	Autologiset glioomasolut hoidettuna antisense-molekyylillä, joka ovat suunnattu insuliinin kaltaisen kasvutekijä 1:den reseptoria kohtaan	Glioman hoito
French	Cellule autologue the Autologous glioma tumour cells treated with antisense molecule directed against the insulin-like growth factor type 1 receptor	Traitement des gliomes
German	Autologe Glioma-Zellen, behandelt mit einem Antisense Molekül gerichtet gegen den Insulin-like Growth Factor 1 Rezeptor	Behandlung von Gliomen
Greek	Αυτόλογα κύτταρα γλοιώματος που έχουν υποστεί επεξεργασία με ένα αντινοηματικό μόριο εναντι του υποδοχέα τύπου 1 του ομοειδούς της ινσουλίνης αυξητικού παράγοντα	Θεραπεία του γλοιώματος
Hungarian	Inzulin-szerű növekedési factor 1-es típusú receptora elleni antiszenz molekulával kezelt autológ glioma tumorsejtek	Glioma kezelése
Italian	Cellule tumorali di glioma autologheo trattatei con molecola antisenso diretta contro il recettore del tipo 1 del fattore di crescita insulino-simile	Trattamento del glioma

[1] At the time of designation

Language	Active ingredient	Indication
Latvian	Autologas gliomas audzēja šūnas, kas apstrādātas ar antisensa molekulu, kas vērsta pret 1. tipa insulīnam līdzīgā augšanas faktora receptoru	Gliomas ārstēšana
Lithuanian	Autologinės gliomos auglio ląstelės, paveiktos priešprasme molekule nukreipta prieš nuo insulino panašaus augimo faktoriaus 1 tipo receptorių	Gliomos gydymas
Maltese	Ċelluli awtologi tat-tumur tat-tip glijoma kkurati b'molekula antisens diretta kontra r-riċettur tal-fattur tat-tkabbir tat-tip 1 li jixbah lill-insulina	Kura tal-glioma
Polish	Autologiczne komórki glejaka poddane działaniu cząsteczki skierowanej przeciwko receptorowi insulinopodobnego czynnika wzrostu typu 1	Leczenie glejaka
Portuguese	Células tumorais de glioma autólogas tratadas com molécula antissense dirigida contra o recetor tipo 1 do fator de crescimento semelhante à insulina	Tratamento do glioma
Romanian	Celule tumorale autologe provenite din gliom tratate cu molecule antisens îndreptate împotriva receptorului pentru factorul de creştere insulin-like de tip 1	Tratamentul gliomului
Slovak	Autológne tumorové gliómové bunky liečené antisense molekulou zacielenou na inzulínu-podobný receptor typu 1 rastového faktora	Liečba gliómu
Slovenian	Avtologne gliomske tumorske celice, obdelane s protismerno molekulo, usmerjeno proti receptorju za insulinu podoben rastni faktor tipa1	Zdravljenje glioma
Spanish	Celulas tumorales de glioma autólogas tratadas con molecula antissense dirigida contra el receptor tipo 1 del factor de creciemento sejante a la insulina	Tratamiento del glioma
Swedish	Autologa gliomtumörceller behandlade med antisensmolkyl mot insulinlik tillväxtfaktorreceptor typ 1	Behandling av gliom
Norwegian	Autologe gliom tumorceller behandlet med antisensemolekyl rettet mot insulinlignende vekstfaktor type 1-reseptor	Behandling av gliom
Icelandic	Samgena tróðæxlisfrumur meðhöndlaðar með andþáttarsameind gegn IGF-1 viðtakanum	Meðferð á glíóma