4 July 2018
EMA/273099/2018

Public summary of opinion on orphan designation

H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O2)-NH2-DOTA-225-Actinium for the treatment of glioma

On 25 May 2018, orphan designation (EU/3/18/2023) was granted by the European Commission to Dr Regenold GmbH, Germany, for H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O_2)-NH_2-DOTA-225-Actinium 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 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 (medicines to treat cancer). Patients also received treatments for the symptoms of glioma, including corticosteroids to reduce pressure within the skull and medicines to prevent seizures.

*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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The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with glioma because early studies showed that patients treated with this medicine lived longer when compared indirectly with results with other medicines. 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 a radiopharmaceutical which contains a radioactive substance (actinium 225). When injected directly into the tumour, the medicine attaches to a receptor (target) called NK-1 on the cancer cells and then enters the cell. Once inside the cell, the radiation emitted by actinium 225 kills the cell, thus slowing 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 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 19 April 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	H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O_2)-NH_2-DOTA-225-actinium	Treatment of glioma
Bulgarian	H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O_2)-NH_2-DOTA-225-актиний	Лечение на глиома
Croatian	H-Arg-Pro-Lis-Pro-Gln-Gln-Fe-2Ti-Gli-Leu-Met(O_2)-NH_2-DOTA-225-aktinij	Liječenje glioma
Czech	H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O_2)-NH_2-DOTA-225-actinium	Léčba gliomů
Danish	H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O_2)-NH_2-DOTA-225-actinium	Behandling af gliom
Dutch	H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O_2)-NH_2-DOTA-225-actinium	Behandeling van glioma
Estonian	H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O_2)-NH_2-DOTA-225-actinium	Glioomi ravi
Finnish	H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O_2)-NH_2-DOTA-225-actinium	Glioman hoito
French	H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O_2)-NH_2-DOTA-225-actinium	Traitement des gliomes
German	H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O_2)-NH_2-DOTA-225-actinium	Behandlung von Gliomen
Greek	H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O_2)-NH_2-DOTA-225-ακτίνιο	Θεραπεία του γλοιώματος
Hungarian	H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O_2)-NH_2-DOTA-225-actinium	Glioma kezelése
Italian	H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O_2)-NH_2-DOTA-225-actinium	Trattamento del glioma
Latvian	H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O_2)-NH_2-DOTA-225-aktīnijs	Gliomas ārstēšana
Lithuanian	H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O_2)-NH_2-DOTA-225-aktiniumas	Gliomos gydymas
Maltese	Attinju H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O_2)-NH_2-DOTA-225	Kura tal-glioma
Polish	H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O_2)-NH_2-DOTA-225-aktyn	Leczenie glejaka
Portuguese	H-Arg-Pro-Lis-Pro-Gln-Gln-Fen-2Tie-Gli-Leu-Met(O_2)-NH_2-DOTA-225-actinio	Tratamento do glioma
Romanian	H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O_2)-NH_2-DOTA-225-actinium	Tratamentul gliomului
Slovak	H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O_2)-NH_2-DOTA-225-actinium	Liečba gliómu

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O_2)-NH_2-DOTA-225-actinium	Zdravljenje glioma
Spanish	H-Arg-Pro-Lis-Pro-Gln-Gln-Phe-2Ti-Gli-Leu-Met(O_2)-NH_2-DOTA-225-actinium	Tratamiento del glioma
Swedish	H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O_2)-NH_2-DOTA-225-actinium	Behandling av gliom
Norwegian	H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O_2)-NH_2-DOTA-225-actinium	Behandling av gliom
Icelandic	H-Arg-Pró-Lýs-Pró-Gln-Gln-Phe-2Thí-Glý-Leu-Met(O_2)-NH_2-DOTA-225-actíníum	Meðferð á glíóma