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Public summary of opinion on orphan designation

Anti-GD2 monoclonal antibody 3F8 humanised for the treatment of neuroblastoma

On 19 November 2018, orphan designation (EU/3/18/2094) was granted by the European Commission to Y-mAbs Therapeutics A/S, Denmark, for anti-GD2 monoclonal antibody 3F8 humanised (also known as naxitamab) for the treatment of neuroblastoma.

What is neuroblastoma?

Neuroblastoma is a cancer of certain nerve cells which is usually seen as a lump in the abdomen or around the spine. Symptoms may include weakness, bone pain, loss of appetite and fever.

Neuroblastoma is the most common solid tumour outside the brain in children. In many cases it is present at birth but is diagnosed later when the cancer has spread to other parts of the body and the child begins to show symptoms of the disease.

Neuroblastoma is a long-term debilitating and life-threatening disease that is associated with poor long-term survival.

What is the estimated number of patients affected by the condition?

At the time of designation, neuroblastoma affected approximately 1.1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 57,000 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 methods were authorised in the EU for the treatment of neuroblastoma, including surgery, chemotherapy (medicines to treat cancer) and radiotherapy (treatment with radiation). The medicine Qarziba (dinutuximab beta) was approved for patients with high-risk neuroblastoma (which has a high chance of coming back).

*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 neuroblastoma. Preliminary data showed that patients responded in a similar way to this medicine and to existing treatments, but infusions with the medicine could be shorter and less frequent.

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) that has been designed to recognise and attach to a specific structure called GD2 that is present in high amounts on the surface of neuroblastoma cells, but not normal cells. When the medicine attaches to the neuroblastoma cells, it is expected to make them a target for the body's immune system (the body's natural defences), which then kills the cancer cells.

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 neuroblastoma were ongoing.

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 18 October 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	Anti-GD2 monoclonal antibody 3F8 humanised	Treatment of neuroblastoma
Bulgarian	Хуманизирано анти-GD2 моноклонално антитяло 3F8	Лечение на невробластом
Croatian	Humanizirano anti-GD2 monoklonalno protutijelo 3F8	Liječenje neuroblastoma
Czech	Anti-GD2 humanizovaná monoklonální protilátka 3F8	Léčba neuroblastomu
Danish	Humaniseret 3F8 Anti-GD2 monoclonalt antistof	Behandling af neuroblastom
Dutch	Anti-GD2 monoclonaal antilichaam 3F8 gehumaniseerd	Behandeling van neuroblastoom
Estonian	GD2-vastane humaniseeritud monoklonaalne antikeha 3F8	Neuroblastoomi ravi
Finnish	Humanisoitu anti-GD2 monoklonaalinen vasta-aine 3F8	Neuroblastooman hoito
French	Anticorps monoclonal humanisé 3F8 anti-GD2	Traitement du neuroblastome
German	Humanisierter anti-GD2 monoklonaler Antikörper 3F8	Behandlung des Neuroblastoms
Greek	Ανθρωποποιημένο αντι-GD2 μονοκλωνικό αντίσωμα 3F8	Θεραπεία του νευροβλαστώματος
Hungarian	GD2-ellenes humanizált 3F8 monoklonális antitest	Neuroblastoma kezelése
Italian	Anticorpo umanizzato 3F8 anti-GD2	Trattamento del neuroblastoma
Latvian	Anti-GD2 humanizēta 3F8 monoklonālā antiviela	Neiroblastomas ārstēšana
Lithuanian	Žmogaus 3F8 monokloninis antikūnas anti-GD2	Neuroblastomos gydymas
Maltese	Antikorp 3F8 monoklonali anti-GD2 umanizzat	Kura tan-newroblastoma
Polish	Humanizowane monoklonalne przeciwciało 3F8 anty-GD2	Leczenie nerwiaka płodowego
Portuguese	Anticorpo monoclonal 3F8 humanizado anti-GD2	Tratamento do neuroblastoma
Romanian	Anticorp monoclonal umanizat 3F8 anti-GD2	Tratamentul neuroblastomului
Slovak	Anti-GD2 monoklonálna protilátka 3F8 humanizovaná	Liečba neuroblastómu
Slovenian	Humanizirano anti-GD2 monoklonsko protitelo 3F8.	Zdravljenje nevroblastoma
Spanish	Anticuerpo monoclonal humanizado de tipo 3F8 anti-GD2	Tratamiento del neuroblastoma
Swedish	Anti-GD2 monoklonal antikropp 3F8 humaniserad	Behandling av neuroblastom
Norwegian	Anti-GD 2 monoklonalt antistoff 3F8 humanisert	Behandling av nevroblastom
Icelandic	Manna einstofna 3F8 mótefni gegn GD2	Meðferð við taugakímfrumuæxli

[1] At the time of designation