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EMA/COMP/300521/2011
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Chimeric monoclonal antibody against GD2 for the treatment of neuroblastoma

On 21 June 2011, orphan designation (EU/3/11/879) was granted by the European Commission to United Therapeutics Europe Ltd, United Kingdom, for chimeric monoclonal antibody against GD2 for the treatment of neuroblastoma.

What is neuroblastoma?

Neuroblastoma is a cancer of nerve cells which is usually seen as a lump in the abdomen (belly area) 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 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 is equivalent to a total of around 56,000 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 neuroblastoma in the EU. Treatments for neuroblastoma included surgery, radiotherapy (treatment with radiation) and chemotherapy (medicines to treat cancer).

The sponsor has provided sufficient information to show that chimeric monoclonal antibody against GD2 might be of significant benefit for patients with neuroblastoma because it works in a different way

*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 Liechtenstein. This represents a population of 506,300,000 (Eurostat 2011).

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to existing treatments, and early studies show that it might improve the outcome of patients with this condition. 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?

Chimeric monoclonal antibody against GD2 is a monoclonal antibody (a type of protein) that has been designed to recognise and attach to a specific structure (an antigen) called GD2. GD2 is a substance that is present in high amounts on the surface of neuroblastoma cells, but not normal cells. The way this medicine works is not fully understood, but studies have shown that in the presence of certain cells or proteins of the body's defence system, the neuroblastoma cells to which the medicine is attached die.

What is the stage of development of this medicine?

The effects of chimeric monoclonal antibody against GD2 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 this medicine had been granted in the United States of America for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 April 2011 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:

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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.

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

Language	Active ingredient	Indication
English	Chimeric monoclonal antibody against GD2	Treatment of neuroblastoma
Bulgarian	Химерно моноклонално антитяло срещу GD2	Лечение на невробластом
Czech	Chimerická monoklonální protilátka proti GD2	Léčba neuroblastomu
Danish	Kimært monoklonalt antistof mod GD2	Behandling af neuroblastom
Dutch	Chimeer monoklonaal antilichaam tegen GD2	Behandeling van neuroblastoom
Estonian	Kimeeriline monoklonaalne antikeha GD2 vastu	Neuroblastoomi ravi
Finnish	Kimeerinen monoklonaalinen vasta-aine GD2:a vastaan	Neuroblastooman hoito
French	Anticorps monoclonal chimérique anti-GD2	Traitement du neuroblastome
German	Chimärer monoklonaler Antikörper gegen GD2	Behandlung des Neuroblastoms
Greek	Χιμαιρικό μονοκλωνικό αντίσωμα έναντι του GD2	Θεραπεία Νευροβλάστωματος
Hungarian	GD2 elleni kiméra monoklonális antitest	Neuroblastoma kezelése
Italian	Anticorpo monoclonale chimerico anti-GD2	Trattamento del neuroblastoma
Latvian	Himēriska monoklonāla antiviela pret GD2	Neiroblastomas ārstēšana
Lithuanian	Chimerinis monokloninis antikūnas prieš GD2	Neuroblastomos gydymas
Maltese	Anti-korp monoklonali kimeriku kontra GD2	Kura tan-newroblastoma
Polish	Chimeryczne przeciwciało monoklonalne skierowane przeciw GD2	Leczenie nerwiaka płodowego
Portuguese	Anticorpo monoclonal quimérico anti-GD2	Tratamento do neuroblastoma
Romanian	Anticorp monoclonal chimeric anti-GD2	Tratamentul neuroblastomului
Slovak	Chimérická monoklonálna protilátka proti GD2	Liečba neuroblastómu
Slovenian	Himerno monoklonsko protitelo proti GD2	Zdravljenje nevroendokrinega nevroblastoma
Spanish	Anticuerpo monoclonal quimérico dirigido contra GD2	Tratamiento del neuroblastoma
Swedish	Chimär monoklonal antikropp mot GD2	Behandling av neuroblastom
Norwegian	Kimært, monoklonalt antistoff mot GD2	Behandling av neuroblastom
Icelandic	Einstofna blendingsmótefni gegn GD2	Meðferð við taugakímfrumuæxli

[1] At the time of designation