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EMA/COMP/292651/2009 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Murine monoclonal antibody to GD2 for the treatment of neuroblastoma

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in January 2011 on request of the Sponsor.

On 12 June 2009, orphan designation (EU/3/09/644) was granted by the European Commission to United Therapeutics Europe Ltd, United Kingdom, for murine monoclonal antibody to GD2 for the treatment of neuroblastoma.

What is neuroblastoma?

Neuroblastoma is the most common solid tumour outside the brain in children. It is often present at birth but is usually 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 cancer of specialised nerve cells in the abdomen (tummy) and manifests itself as a lump in the abdomen or around the spine. Other symptoms may include weakness, bone pain, loss of appetite and fever.

Neuroblastoma is a life-threatening disease of childhood that leads to 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 threshold 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 (using radiation to kill the cancer cells) and chemotherapy (medicines to treat cancer).

*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 504,800,000 (Eurostat 2009).

The sponsor has provided sufficient information to show that murine monoclonal antibody to GD2 might be of significant benefit for patients with neuroblastoma because it works in a different way to existing medicines and might represent an alternative way to treat this disease. 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?

Murine monoclonal antibody to 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 the complement system, which is part 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 murine monoclonal antibody to GD2 have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with neuroblastoma were ongoing.

At the time of submission, murine monoclonal antibody to GD2 was not authorised anywhere in the EU for neuroblastoma. Orphan designation of this medicine had been granted in the United States of America for neuroblastoma.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 5 May 2009 recommending the granting of this designation.

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Murine monoclonal antibody to GD2	Treatment of neuroblastoma
Bulgarian	Мише моноклонално антитяло срещу GD2	Лечение на невробластом
Czech	Myší monoklonální protilátka proti GD2	Léčba neuroblastomu
Danish	Murint monoklonalt antistof mod GD2	Behandling af neuroblastom
Dutch	Murien monoklonaal antilichaam tegen GD2	Behandeling van neuroblastoom
Estonian	Hiire monoklonaalne antikeha GD2 vastu	Neuroblastoomi ravi
Finnish	Hiiren monoklonaalinen anti-GD2-vasta-aine	Neuroblastooman hoito
French	Anticorps monoclonal murin anti-GD2	Traitement du neuroblastome
German	Muriner monoklonaler anti-GD2-Antikörper	Behandlung des Neuroblastoms
Greek	Μονοκλωνικό αντίσωμα τρωκτικού κατά του GD2	Θεραπεία Νευροβλάστωματος
Hungarian	GD2 elleni egér/patkány monoklonális antitest	Neuroblastoma kezelése
Italian	Anticorpo monoclonale murino anti GD2	Trattamento del neuroblastoma
Latvian	Peļu monoklonālās antivielas pret GD2	Neiroblastomas ārstēšana
Lithuanian	Pelės monokloninis antikūnas prieš GD2	Neuroblastomos gydymas
Maltese	Antikorp monoklonali tal-ġrieden għall-GD2	Kura tan-newroblastoma
Polish	Mysie przeciwciało monoklonalne przeciw GD2	Leczenie nerwiaka płodowego
Portuguese	Anticorpo monoclonal murino anti-GD2	Tratamento do neuroblastoma
Romanian	Anticorp monoclonal murin anti GD2	Tratamentul neuroblastomului
Slovak	Myšia monoklonálna protilátka proti GD2	Liečba neuroblastómu
Slovenian	Mišje monoklonsko protitelo proti antigenu GD2	Zdravljenje nevroendokrinega nevroblastoma
Spanish	Anticuerpo monoclonal murino anti-GD2	Tratamiento del neuroblastoma
Swedish	Murin monoklonal antikropp mot GD2	Behandling av neuroblastom
Norwegian	Murint monoklonalt antistoff mot GD2	Behandling av neuroblastom
Icelandic	Músa einstofna mótefni gegn GD2	Meðferð við taugakímfrumuæxli

¹ At the time of designation