2 March 2015
EMA/COMP/786549/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Chimeric monoclonal antibody to O-acetyl-GD2 antigen for the treatment of neuroblastoma

On 15 January 2015, orphan designation (EU/3/14/1416) was granted by the European Commission to Atlab Pharma SAS, France, for chimeric monoclonal antibody to O-acetyl-GD2 antigen 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 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 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 in the EU for the treatment of neuroblastoma. Treatments for neuroblastoma included surgery, chemotherapy (medicines to treat cancer) and radiotherapy (treatment with radiation).

*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 511,100,000 (Eurostat 2014).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

The sponsor has provided sufficient information to show that chimeric monoclonal antibody to O-acetyl-GD2 antigen might be of significant benefit for patients with neuroblastoma because it works in a different way to currently authorised treatments by specifically targeting tumour tissue. In addition, early studies in experimental models showed improved anti-tumour activity. These assumptions 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 to O-acetyl-GD2 antigen is a monoclonal antibody (a type of protein) that has been designed to recognise and attach to a specific structure (an antigen) called O-acetyl-GD2. O-acetyl-GD2 is a substance 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 marks them out as a target for the body's immune system, which is then expected to attack the cancer cells and thereby reverse or slow down the progression of the disease.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of chimeric monoclonal antibody to O-acetyl-GD2 antigen in experimental models was ongoing.

At the time of submission, no clinical trials with the medicine in patients with neuroblastoma had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for neuroblastoma 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 11 December 2014 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:

Atlab Pharma SAS
3 chemin du Pressoir Chênaie
44100 Nantes
France
Tel. +33 2 28 08 00 31
E-mail: info@atlab-pharma.com

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 to O-acetyl-GD2 antigen	Treatment of neuroblastoma
Bulgarian	Химерично моноклонално антитяло, специфично за О-ацетил-GD2 Антиген	Лечение на невробластом
Croatian	Kimerično monoklonsko protutijelo specifično za antigen O-acetil-GD2	Liječenje neuroblastoma
Czech	Chimérická monoklonální protilátka vůči antigenu O-acetyl-GD2	Léčba neuroblastomu
Danish	Kimerisk monoklonalt antistof specifikt for O-Acetyl-GD2 antigen	Behandling af neuroblastom
Dutch	Chimeer monoklonaal antilichaam voor O-acetyl-GD2 antigen	Behandeling van neuroblastoom
Estonian	O-atsetüül-GD2 antikehale spetsiifiline kimäärne monoklonaalne antigeen	Neuroblastoomi ravi
Finnish	O-asetyyli-GD2-antigeeni-spesifinen kimeerinen monoklonaalinen vasta-aine	Neuroblastooman hoito
French	Anticorps monoclonal chimérique spécifique de l'antigène GD2-O acétylé	Traitement du neuroblastome
German	Chimärer monoklonaler Antikörper, spezifisch für das O-Acetyl-GD2-Antigen	Behandlung des Neuroblastoms
Greek	Χιμαιρικό μονοκλωνικό αντίσωμα για το O-Ακετυλο-GD2 αντιγόνο	Θεραπεία του νευροβλαστώματος
Hungarian	O-acetil-GD2 antitestre specifikus kimerikus monoklonális antitest	Neuroblastoma kezelése
Italian	Anticorpo monoclonale chimerico specifico per l'antigene anti-O-acetil-GD2	Trattamento del neuroblastoma
Latvian	Himēriska monoklonāla antiviela pret O-acetil-GD2 antigēnu	Neiroblastomas ārstēšana
Lithuanian	Chimerinis monokloninis antikūnas prieš O-acetil-GD2 antigeną	Neuroblastomos gydymas
Maltese	Antikorp monoklonali kimeriku għall-antiġen O-acetyl-GD2	Kura tan-newroblastoma
Polish	Chimeryczne przeciwciało monoklonalne swoiste dla szpiczaka O-acetylowanej formy GD2	Leczenie nerwiaka płodowego
Portuguese	Anticorpo monoclonal quimérico contra o antigénio O-acetil-GD2	Tratamento do neuroblastoma
Romanian	Anticorp monoclonal chimeric specific antigenului O-Acetil-GD2	Tratamentul neuroblastomului
Slovak	Chimerická monoklonálna protilátka špecifická k antigénu O-acetyl-GD2	Liečba neuroblastómu

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Himerno monoklonalno protitelo, specifično za antigen O-acetil GD2	Zdravljenje nevroendokrinega nevroblastoma
Spanish	Anticuerpo monoclonal quimérico específico para el antígeno contra O-acetil-GD2	Tratamiento del neuroblastoma
Swedish	Kimär monoklonal antikropp som är specifik mot O-acetyl-GD2-antigenet	Behandling av neuroblastom
Norwegian	Kimerisk monoklonalt antistoff spesifikt for O-acetyl-GD2-antigen	Behandling av nevroblastom
Icelandic	Blendings einstofna mótefni, sértækt fyrir O-acetýl-GD2-mótefnavaka	Meðferð við taugakímfrumuæxli