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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

7-Beta-hydroxy cholesteryl-3-beta-oleate for the treatment of glioma

On 17 December 2010, orphan designation (EU/3/10/816) was granted by the European Commission to Intsel Chimos SA, France, for 7-beta-hydroxy cholesteryl-3-beta-oleate 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 experienced 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 a fifth of patients with glioma have seizures (fits) for months or years before the disease is diagnosed.

Glioma is a debilitating and life-threatening disease because of the severe damage to the brain that 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 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 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 27), Norway, Iceland and Liechtenstein. This represents a population of 506,500,000 (Eurostat 2010).

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The sponsor has provided sufficient information to show that 7-beta-hydroxy cholesteryl-3-beta-oleate might be of significant benefit for patients with glioma because it works in a different way to existing treatments and early studies in experimental models show that it might improve the treatment of patients with this condition. 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?

7-Beta-hydroxy cholesteryl-3-beta-oleate is an oxysterol (a substance derived from cholesterol, a fat commonly found in the body). The substance is expected to work by blocking the action of some enzymes, known as protein kinases B (or Akt), in the glioma cells. These enzymes are involved in stimulating cells to grow and divide. As tumour cells grow and divide uncontrollably, the medicine is expected to help control the growth and division of the tumour cells and slow down the development of the glioma.

7-Beta-hydroxy cholesteryl-3-beta-oleate is going to be injected directly into the brain tumour contained within microscopic fat particles called 'liposomes', which are expected to carry the medicine into the glioma cells.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, studies with 7-beta-hydroxy cholesteryl-3-beta-oleate in experimental models were ongoing.

At the time of submission, clinical trials with 7-beta-hydroxy cholesteryl-3-beta-oleate in patients with gliomas were ongoing.

At the time of submission, 7-beta-hydroxy cholesteryl-3-beta-oleate 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 7 October 2010 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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	7-beta-hydroxycholesteryl-3-beta-oleate	Treatment of glioma
Bulgarian	7-бета-хидрокси холестерил-3-бета-олеат	Лечение на глиома
Czech	7-beta-hydroxy-Cholesteryl-3-beta-oleát	Léčba gliomů
Danish	7-beta-hydroxycholesteryl-3-beta-oleat	Behandling af gliom
Dutch	7-beta-hydroxycholesteryl-3-beta-oleaat	Behandeling van glioma
Estonian	7-beta-hüdroksükolesterüül-3-beta-oleaat	Glioomi ravi
Finnish	7-beta-hydroksikolesteryyli-3-beta-oleaatti	Glioman hoito
French	7-beta-hydroxy cholesteryl-3-beta-oleate	Traitement des gliomes
German	7-beta-hydroxycholesteryl-3-beta-oleat	Behandlung von Gliomen
Greek	7-β-υδροξυ χοληστερολη-3-β-ελαϊκή	Θεραπεία του γλοιώματος
Hungarian	7-beta-hidroxi koleszteril-3-beta-oleát	Glioma kezelése
Italian	7-beta-idrossi colesteril-3-beta-oleato	Trattamento del glioma
Latvian	7-beta-hidroksi-holesteril-3-beta-oleāts	Gliomas ārstēšana
Lithuanian	7-beta-hidroksicholesteril-3-beta-oleatas	Gliomos gydymas
Maltese	7-beta-hydroxy cholesteryl-3-beta-oleate	Kura tal-glioma
Polish	3-beta-oleinian 7-beta-hydroksycholesterolu	Leczenie glejaka
Portuguese	7-beta-hidroxi-colesteril-3-beta-oleato	Tratamento do glioma
Romanian	7-beta-hidroxicolesteril-3-beta-oleat	Tratamentul gliomului
Slovak	7-beta-hydroxycholesteryl-3-beta-oleát	Liečba gliómu
Slovenian	7-beta-hidroksi holesteril-3-beta-oleat	Zdravljenje glioma
Spanish	7-beta-hidroxi-3-colesteril oleato-beta	Tratamiento del glioma
Swedish	7-beta-hydroxykolesteryl-3-beta-oleat	Behandling av gliom
Norwegian	7-beta-hydroksykolesteryl-3-beta-oleat	Behandling av gliom
Icelandic	7-beta-hýdroxýkólesterýl-3-beta-óleat	Meðferð á glíóma

[1] At the time of designation