



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

2-hydroxyoleic acid for the treatment of glioma

On 27 October 2011, orphan designation (EU/3/11/916) was granted by the European Commission to Lipopharma Therapeutics SL, Spain, for 2-hydroxyoleic acid 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 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 51,000 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 glioma in the EU. Treatments for glioma included surgery and radiotherapy (treatment with radiation), as well as chemotherapy (medicines to treat cancer) to improve survival. 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 506,300,000 (Eurostat 2011).



The sponsor has provided sufficient information to show that 2-hydroxyoleic acid might be of significant benefit for patients with glioma because it works in a different way to existing treatments and early studies 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?

2-hydroxyoleic acid is a fatty acid, similar to oleic acid, which is found in animal and vegetable fats. It is expected to work in glioma by entering the membrane of the glioma cells (which contains many other types of fatty acids), and changing its composition in a way that stops a protein called 'Ras' attaching to it. The Ras protein needs to attach to the cell membrane in order to activate a chain of reactions to stimulate cell growth and division. When it is blocked, this is expected to slow the growth and spread of the cancer cells.

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 2-hydroxyoleic acid in experimental models was ongoing.

At the time of submission of the application for orphan designation, no clinical trials with 2-hydroxyoleic acid in patients with glioma had been started.

At the time of submission, 2-hydroxyoleic acid 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 8 September 2011 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	2-hydroxyoleic acid	Treatment of glioma
Bulgarian	2-хидрокси олеинова киселина	Лечение на глиома
Czech	2-hydroxyolejová kyselina	Léčba gliomů
Danish	2-hydroxyoliesyre	Behandling af gliom
Dutch	2-hydroxyolëiczuur	Behandeling van glioma
Estonian	2-hüdroksüoleiinhape	Glioomi ravi
Finnish	2-hydroksi-oleiinihappo	Gliooman hoito
French	Acide 2-Hydroxyoléique	Traitement des gliomes
German	2-Hydroxy-Ölsäure	Behandlung von Gliomen
Greek	2- υδροξυ ολεικό οξύ	Θεραπεία του γλοιώματος
Hungarian	2-hidroxi-olajsav	Glioma kezelése
Italian	Acido 2-idrossioleico	Trattamento del glioma
Latvian	2-hidroksioelīnskābe	Gliomas ārstēšana
Lithuanian	2-hidroksioleino rūgštis	Gliomos gydymas
Maltese	2-hydroxyoleic acid	Kura tal-glioma
Polish	Kwas 2-hydroksyoleinowy	Leczenie glejaka
Portuguese	Ácido 2-hidroxioléico	Tratamento do glioma
Romanian	Acid 2-hidroxioléic	Tratamentul gliomului
Slovak	Kyselina 2-hydroxyolejová	Liečba gliómu
Slovenian	2-hidroksioleinska kislina	Zdravljenje glioma
Spanish	Ácido 2-hidroxioléico	Tratamiento del glioma
Swedish	2-hydroxy-oljesyra	Behandling av gliom
Norwegian	2-hydroksyoljesyre	Behandling av gliom
Icelandic	2-hýdróxiólíusýra	Meðferð á glíóma

¹ At the time of designation