



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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EMA/COMP/848/2013  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### 1,2:5,6-Dianhydrogalactitol for the treatment of glioma

On 24 January 2013, orphan designation (EU/3/12/1093) was granted by the European Commission to IDIS Ltd, United Kingdom, for 1,2:5,6-dianhydrogalactitol 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 long-term debilitating and life-threatening disease because of the severe damage to the brain, and 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 2.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 112,000 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, radiotherapy (treatment with radiation), and 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.

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\*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 509,000,000 (Eurostat 2013).



The sponsor has provided sufficient information to show that 1,2:5,6-dianhydrogalactitol might be of significant benefit for patients with glioma because early studies show that it might improve the treatment 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?**

1,2:5,6-Dianhydrogalactitol is a small molecule that can easily cross the blood-brain barrier that separates the blood from the brain tissue. It is expected to work as an 'alkylating agent'. This means that it attaches a chemical group called an 'alkyl' group to DNA of cells while they are reproducing. This damages the DNA and in turn disrupts cell division. As a result, the tumour cells in the brain cannot reproduce, and this slows down the growth of the glioma.

### **What is the stage of development of this medicine?**

The effects of 1,2:5,6-dianhydrogalactitol have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with 1,2:5,6-dianhydrogalactitol in patients with glioma were ongoing.

At the time of submission, 1,2:5,6-dianhydrogalactitol was not authorised anywhere in the EU for glioma. Orphan designation of 1,2:5,6-dianhydrogalactitol 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 6 December 2012 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:

IDIS Ltd  
IDIS House  
Churchfield Road  
Weybridge KT13 8DB  
United Kingdom  
Telephone: +44 1932 824 000  
Telefax: +44 1932 824 200  
E-mail: [gma@idispharma.com](mailto:gma@idispharma.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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	1,2:5,6-Dianhydrogalactitol	Treatment of glioma
Bulgarian	1,2:5,6-дианхидрогалактитол	Лечение на глиома
Czech	1,2:5,6- Dianhydrogalactitol	Léčba gliomů
Danish	1,2:5,6-dianhydrogalactitol	Behandling af gliom
Dutch	1,2:5,6-Dianhydrogalactitol	Behandeling van glioma
Estonian	1,2:5,6-dianhüdrogalaktitool	Glioomi ravi
Finnish	1,2:5,6-Dianhydrogalaktitoli	Gliooman hoito
French	1,2:5,6-Dianhydrogalactitol	Traitement des gliomes
German	1,2:5,6-Dianhydrogalactitol	Behandlung von Gliomen
Greek	1,2:5,6 Διανυδρογαλακτιτόλη	Θεραπεία του γλοιώματος
Hungarian	1,2:5,6-Dianhidrogalaktitol	Glioma kezelése
Italian	1,2:5,6-Dianidrogalactitolo	Trattamento del glioma
Latvian	1,2:5,6 Dianhidrogalaktitols	Gliomas ārstēšana
Lithuanian	1,2:5,6 - dianhidrogalaktitolis	Gliomos gydymas
Maltese	1,2:5,6-Dianhydrogalactitol	Kura tal-glioma
Polish	1,2:5,6-Dianhydrogalaktytol	Leczenie glejaka
Portuguese	1,2:5,6-Dianidrogalactitol	Tratamento do glioma
Romanian	1,2:5,6 Dianhidrogalactitol	Tratamentul gliomului
Slovak	1,2:5,6-Dianhydrogalaktitol	Liečba gliómu
Slovenian	1,2:5,6-Dianhydrogalactitol	Zdravljenje glioma
Spanish	1,2:5,6 Dianhydrogalactitol	Tratamiento del glioma
Swedish	1,2:5,6-Dianhydrogalaktitol	Behandling av gliom
Norwegian	1,2:5,6-Dianhydrogalaktitol	Behandling av gliom
Icelandic	1,2:5,6-Díanhýdrógalaktitól	Meðferð á glíóma

<sup>1</sup> At the time of designation