



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

2 July 2012
EMA/COMP/285075/2012
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

N-Hydroxy-4-(3-methyl-2-(S)-phenyl-butyrylamino) benzamide for the treatment of meningioma

On 6 June 2012, orphan designation (EU/3/12/996) was granted by the European Commission to Sirius Regulatory Consulting Limited, United Kingdom, for N-hydroxy-4-(3-methyl-2-(S)-phenyl-butyrylamino) benzamide for the treatment of meningioma.

What is meningioma?

Meningioma is a type of benign (noncancerous) slow-growing brain tumour that affects the meninges, the protective membranes around the brain and spinal cord. Meningiomas occur most commonly in women over 60 years of age. The main symptoms of the disease arise from the increased pressure within the skull and include headaches, vomiting, problems with vision and coordination, changes in personality, memory loss and seizures (fits).

Meningioma is a debilitating disease because of the damage caused by the tumour in the brain and spinal cord. It can also be life threatening due to the risk of the tumour becoming cancerous.

What is the estimated number of patients affected by the condition?

At the time of designation, meningioma affected not more than 1.5 in 10,000 people in the European Union (EU)*. This is equivalent to a total of not more than 76,000 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, the main treatment for meningioma was surgery to remove the tumour. Surgery can cure the disease in the majority of cases.

The sponsor has provided sufficient information to show that N-hydroxy-4-(3-methyl-2-(S)-phenyl-butyrylamino) benzamide might be of significant benefit for patients with meningioma because early

*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).



studies in experimental models show that it may decrease tumour growth, therefore improving the treatment of patients with this condition, particularly patients whose disease has come back after surgery or for whom surgery is not an option. 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?

This medicine is expected to reduce tumour formation mainly by blocking the activity of histone deacetylases (HDACs), proteins that promote cell division. By blocking HDACs, the medicine is expected to stop cells from dividing and multiplying, leading to cell death. The medicine may also work through other means such as damaging the internal skeleton of the tumour cells and blocking the ability of the cells to repair their DNA.

What is the stage of development of this medicine?

The effects of N-hydroxy-4-(3-methyl-2-(S)-phenyl-butyrylamino) benzamide have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with meningioma had been started.

At the time of submission, this medicine was not authorised anywhere in the EU for meningioma. Orphan designation of this medicine 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 12 April 2012 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	N-hydroxy-4-(3-methyl-2-(S)-phenyl-butrylamino) benzamide	Treatment of meningioma
Bulgarian	N-хидрокси-4-(3-метил-2-(S)-фенил-бутириламино) бензамид benzamide	Лечение на менингиом
Czech	N-hydroxy-4-(3-methyl-2-(S)-fenyl-butrylamino) benzamid	Léčba meningeomu
Danish	N-hydroxy-4-(3-methyl-2-(S)-phenyl-butrylamino) benzamid	Behandling af meningeom
Dutch	N-hydroxy-4-(3-methyl-2-(S)-fenyl-butrylamino) benzamide	Behandeling van meningeoom
Estonian	N-hüdoksü-4-(3-metüül-2-(S)-fenüül-butrylamino) bensamiid	Meningioomi ravi
Finnish	N-hydroksi-4-(3-metyyli-2-(S)-fenyli-butrylamino) bentsamidi	Meningeooman hoito
French	N-hydroxy-4-(3-méthyl-2-(S)-phényl-butrylamino) benzamide	Traitement des Méningiomes
German	N-Hydroxy-4-(3-methyl-2-(S)-phenyl-Butrylamino) benzamid	Behandlung von Meningeomen
Greek	N-υδροξυ-4-(3-μεθυλο-2-(S)-φαινυλο-βουτυριλαμινο) βενζαμιδιό	Θεραπεία του μηνιγγιώματος
Hungarian	N-hidroxi-4-(3-metil-2-(S)-fenil-butrylamino) benzamide	Meningeoma kezelése
Italian	N-idrossi-4-(3-metil-2-(S)-fenil-butrylamino) benzamide	Trattamento del Meningioma
Latvian	N-hidroksi-4-(3-metil-2-(S)-fenil-butirilamino) benzamīds	Meningiomas ārstēšana
Lithuanian	N-hidroksi-4-(3-metil-2-(S)-fenil-butirilamino) benzamidas	Meningiomos gydymas
Maltese	N-hydroxy-4-(3-metil-2-(S)-fenil-butrylamino) benzamide	Kura tal-meningoma
Polish	N-hydroksy-4-(3-metylo-2-(S)-fenylo-butrylamino) benzamid	Leczenie Oponiaka
Portuguese	N-hidróxi-4-(3-metil-2-(S)-fenil-butrylamino) benzamida	Tratamento do Meningioma
Romanian	N-hidroxi-4-(3-metil-2-(S)-fenil-butirilamino) benzamidă	Tratamentul meningiomului
Slovak	N-hydroxy-4-(3-metyl-2-(S)-fenyl-butrylamino) benzamid	Liečba meningeómu
Slovenian	N-hidroksi-4-(3-metil-2-(S)-fenil-butirilamino) benzamid	Zdravljenje meningioma
Spanish	N-hidroxi-4-(3-metil-2-(S)-fenil-butirilamino) benzamida	El tratamiento del meningioma
Swedish	N-hydroxi-4-(3-metyl-2-(S)-fenyl-butrylamino) bensamid	Behandling av meningiom
Norwegian	N-hydroksy-4-(3-metyl-2-(S)-fenyl-butrylamino) benzamid	Behandling av meningeom
Icelandic	N-hýdroxý-4-(3-metýl-2-(S)-fenýl-bútýrýlaminó) benzamíð	Meðferð við meningióma

¹ At the time of designation