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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Eflornithine for the treatment of neuroblastoma

On 27 September 2011, orphan designation (EU/3/11/902) was granted by the European Commission to Cancer Prevention Pharma Ltd, UK, for eflornithine 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 (belly area) 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 is 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).

The sponsor has provided sufficient information to show that eflornithine might be of significant benefit for patients with neuroblastoma because it works in a different way to existing treatments, and early studies show that it might improve the outcome of patients with this condition, possibly in combination

*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).

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

with existing treatments. 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?

Eflornithine works by blocking the action of an enzyme called ornithine decarboxylase, a polyamine enzyme, which is involved in the production of polyamines, substances required for cells to grow. In some patients with neuroblastoma, polyamine enzymes are over-activated and this has been linked with the rapid growth of cancer cells. By blocking ornithine decarboxylase, eflornithine is expected to slow down the growth of the cancer cells. This medicine is expected to be available as tablets and as a powder to be taken by mouth.

What is the stage of development of this medicine?

The effects of eflornithine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with eflornithine in patients with neuroblastoma were ongoing.

At the time of submission, eflornithine was not authorised anywhere in the EU for neuroblastoma. Orphan designation of eflornithine had been granted in the United States for neuroblastoma.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 July 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

Cancer Prevention Pharma Limited
c/o Arnold & Porter UK LLP
Tower 42
25 Old Broad Street
London EC2N 1HQ
United Kingdom
Telephone: + 44 20 7786 61 00
Telefax: + 44 20 7786 62 99

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	Eflornithine	Treatment of neuroblastoma
Bulgarian	Ефлорнитин	Лечение на невробластом
Czech	Eflornithin	Léčba neuroblastomu
Danish	Eflornithin	Behandling af neuroblastom
Dutch	Eflornithine	Behandeling van neuroblastoom
Estonian	Eflornitiin	Neuroblastoomi ravi
Finnish	Eflornitiini	Neuroblastooman hoito
French	Eflornithine	Traitement du neuroblastome
German	Eflornithin	Behandlung des Neuroblastoms
Greek	Εφλορνιθίνη	Θεραπεία Νευροβλάστωματος
Hungarian	Eflornitin	Neuroblastoma kezelése
Italian	Eflornitina	Trattamento del neuroblastoma
Latvian	Eflornitīns	Neiroblastomas ārstēšana
Lithuanian	Eflornitinas	Neuroblastomos gydymas
Maltese	Eflornithine	Kura tan-newroblastoma
Polish	Eflornityna	Leczenie nerwiaka płodowego
Portuguese	Eflornitina	Tratamento do neuroblastoma
Romanian	Eflornitină	Tratamentul neuroblastomului
Slovak	Eflornitín	Liečba neuroblastómu
Slovenian	Eflornitin	Zdravljenje nevroendokrinega nevroblastoma
Spanish	Eflornitina	Tratamiento del neuroblastoma
Swedish	Eflornitin	Behandling av neuroblastom
Norwegian	Eflornitin	Behandling av neuroblastom
Icelandic	Eflornithín	Meðferð við taugakímfrumuæxli

[1] At the time of designation