



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Amrubicin hydrochloride for the treatment of small-cell lung cancer

On 2 April 2008, orphan designation (EU/3/08/538) was granted by the European Commission to Pharmion Ltd, United Kingdom, for amrubicin hydrochloride for the treatment of small-cell lung cancer.

The sponsorship was transferred to Celgene Europe Limited, United Kingdom, in December 2008.

What is small-cell lung cancer?

Small-cell lung cancer is a disease in which cancer cells develop in the lungs. This type of lung cancer accounts only for about 20% of all lung cancer cases. Usually it develops in the central part of the lungs, and the cancer cells are typically small compared with the other types of lung cancer. Signs of cancer are difficult to detect in early stages of the disease, and the majority of the patients are diagnosed when the disease has spread and cannot be surgically removed. Small-cell lung cancer is a life-threatening disease.

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

At the time of designation, small-cell lung cancer affected less than 1.5 in 10,000 people in the European Union (EU)*. This is equivalent to a total of fewer than 75,000 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 knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

The main treatment of small-cell lung cancer consists of chemotherapy (i.e drugs that kill cancer cells) and radiotherapy (i.e using high-dose x-rays or other high-energy rays to kill cancer cells).

Several medicinal products were authorised in the European Community, for the treatment of small-cell lung cancer, at the time of submission of the application for orphan designation. Amrubicin hydrochloride might be of potential significant benefit for the treatment of small-cell lung cancer,

* 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 498,000,000 (Eurostat 2006).



because it might improve the long-term outcome of the patients. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

Amrubicin hydrochloride is a synthetic anthracycline, a type of chemotherapy. Within the tumour, amrubicin hydrochloride is transformed into a substance called amrubicinol. Amrubicin hydrochloride and amrubicinol both stop the production of new genetic material and can therefore kill dividing cells. As tumour cells divide very rapidly, the product is thought to decrease tumour growth through its action on dividing cells.

What is the stage of development of this medicine?

The effects of amrubicin hydrochloride were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with small-cell lung cancer were ongoing and some were completed.

Amrubicin hydrochloride was authorised in Japan for the treatment of both small-cell lung cancer and non-small cell lung cancer, at the time of submission.

Orphan designation of amrubicin hydrochloride was not granted anywhere worldwide for small-cell lung cancer.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 6 February 2008 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	Amrubicin	Treatment of small cell lung cancer
Bulgarian	Амрубицин	лечение за дребноклетъчен белодробен карцином
Czech	Amrubicin	Léčba malobuněčného karcinomu plic
Danish	Amrubicin	Behandling af småcellet lungecancer
Dutch	Amrubicin	Behandeling van kleincellig longcarcinoom
Estonian	Amrubitsiin	Väikeserakulise kopsuvähi ravi.
Finnish	Amrubisiini	Keuhkojen pienisolusyövän hoito
French	Amrubicine	Traitement du cancer du poumon à petites cellules
German	Amrubicin	Behandlung des kleinzelligen Lungenkarzinoms
Greek	Αμρουβικίνη	Θεραπεία του μικροκυτταρικού καρκίνου του πνεύμονα
Hungarian	Amrubicin	Kissejtes tüdőrák kezelése
Italian	Amrubicina	Trattamento del cancro al polmone a piccole cellule
Latvian	Amrubicīns	Sīkšūnu plaušu vēža ārstēšana
Lithuanian	Amrubicinas	Smulkialąstelinio plaučių vėžio gydymas
Maltese	Amrubicin	trattament taċ-ċelluli żgħar tal-kanċer tal-pulmun
Polish	Amrubicyna	Leczenie raka drobnokomórkowego płuc
Portuguese	Amrubicin	Tratamento do carcinoma de pequenas células do pulmão
Romanian	Amrubicină	tratarea cancerului pulmonar microcelular
Slovak	Amrubicin	Liečba malobunkového karcinómu pľúc
Slovenian	Amrubicin	Zdravljenje drobnocelicnega raka pljuč
Spanish	Amrubicina	Carcinoma de pulmón de células pequeñas
Swedish	Amrubicin	Behandling av småcellig lungcancer
Norwegian	Amrubicin	Behandling av småcellet lungekreft
Icelandic	Amrúbísín	Til meðferðar við lungnakrabbameini af smáfrumugerð

¹ At the time of designation