



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Pemetrexed disodium for the treatment of malignant mesothelioma

***Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in June 2004 on request from the sponsor.***

On 17 September 2001, orphan designation (EU/3/01/060) was granted by the European Commission to Eli Lilly Nederland B.V, Netherlands, for pemetrexed disodium for the treatment of malignant mesothelioma.

#### **What is malignant mesothelioma?**

Malignant mesothelioma is a cancer of the membrane that surrounds the lungs (the pleura) or, less commonly, of the membrane that lines the abdomen (the peritoneum). 'Malignant' describes that the cancer is severe and likely to spread easily to other parts of the body. Mesothelioma is a very rare disease, but has become more frequent over the last few decades. It occurs more often in men than in women. Although it can occur at any time of life, it usually occurs at an age of around 60 years.

The major cause of mesothelioma is thought to be exposure to asbestos. Approximately eight out of ten people with mesothelioma have been exposed to asbestos in the past, typically 30 to 40 years before the cancer develops. Malignant mesothelioma is a life-threatening disease.

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

At the time of designation, malignant mesothelioma affected approximately 0.1 in 10,000 people in the European Union (EU)\*. This is equivalent to a total of around 3,800 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).

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\*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union. This represents a population of 377,000,000 (Eurostat 2001).



## **What treatments are available?**

No satisfactory methods for treatment of malignant mesothelioma were authorised at the time of application.

In some cases surgical removal of the tumour is used. However, this is not always possible because the tumour has often already spread to other parts of the body by the time it is diagnosed.

## **How is this medicine expected to work?**

Pemetrexed disodium is a cytotoxic medicine (a medicine that kills cells that are dividing, such as cancer cells), which belongs to the group 'anti-metabolites'. In the body, pemetrexed is converted into an active form that blocks the activity of the enzymes that are involved in producing 'nucleotides' (the building blocks of DNA and RNA, the genetic material of cells). As a result, the active form of pemetrexed slows down the formation of DNA and RNA and prevents the cells from dividing. The conversion of pemetrexed into its active form occurs more readily in cancer cells than in normal cells, leading to higher levels of the active form of the medicine and a longer duration of action in cancer cells. This results in the division of cancer cells being reduced, while normal cells are only slightly affected.

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

The effects of pemetrexed disodium were evaluated in experimental models.

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

Pemetrexed disodium was not marketed anywhere worldwide for the treatment of malignant mesothelioma or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 18 July 2001 recommending the granting of this designation.

Update: Pemetrexed disodium (Alimta) has been authorised in the EU since 22 September 2004. Alimta in combination with cisplatin is indicated for the treatment of chemotherapy naïve patients with unresectable malignant pleural mesothelioma.

More information on Alimta can be found in the European public assessment report (EPAR) on the Agency's website: [ema.europa.eu/Find\\_medicine/Human\\_medicines/European\\_Public\\_Assessment\\_Reports](http://ema.europa.eu/Find_medicine/Human_medicines/European_Public_Assessment_Reports)

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 languages<sup>1</sup>, Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Pemetrexed disodium	Treatment of malignant mesothelioma
Danish	Pemetrexed dinatrium	Behandling af malignt mesoteliom
Dutch	Dinatrium pemetrexed	Behandeling van een maligne mesothelioma
Finnish	Pemetreksididinatrium	Pahanlaatuisen mesoteliooman hoito
French	Pemetrexed Disodique	Traitement du mésothéliome malin
German	Dinatriumpemetrexed	Behandlung des malignen Mesothelioms
Greek	Πιμετριξιδή δινατριούχος	Θεραπεία του κακοήθους μεσοθηλιώματος
Italian	Pemetrexed bisodico	Trattamento del mesotelioma maligno
Potuguese	Pemetrexed dissódico	Tratamento do mesotelioma maligno
Spanish	Pemetrexed disódico	Tratamiento del mesotelioma maligno
Swedish	Dinatriumpemetrexed	Behandling av malignt mesoteliom

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