



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Milciclib maleate for treatment of malignant thymoma

On 8 November 2012, orphan designation (EU/3/12/1059) was granted by the European Commission to Nerviano Medical Science Srl, Italy, for milciclib maleate for the treatment of malignant thymoma.

What is malignant thymoma?

Malignant thymoma is a cancer of the thymus, a gland below the breastbone that is involved in producing infection-fighting cells. Malignant thymoma is most often seen in middle-aged or older people. There may be no symptoms, but some patients have cough, chest pain and difficulty breathing. Up to about half of all patients with malignant thymoma also have myasthenia gravis, a disease causing muscle weakness.

Malignant thymoma is a long-term debilitating and potentially life-threatening illness that is associated with poor long-term survival.

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

At the time of designation, malignant thymoma affected approximately 0.12 in 10,000 persons in the European Union (EU). This was equivalent to a total of approximately 6,100 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, no satisfactory treatment had been authorised in the EU for patients affected by the condition. Treatments for malignant thymoma may include surgery, radiotherapy (treatment with radiation), and chemotherapy (medicines to treat 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. At the time of designation, this represented a population of 509,000,000 (Eurostat 2012).



How is this medicine expected to work?

Milciclib maleate blocks the action of specific enzymes called cyclin-dependent kinases (CDKs), which are involved in cell division. In order to become active, CDKs have to attach to proteins called cyclins. Because cancer cells contain many of these cyclins, CDKs become abnormally active leading to the rapid growth of cancer cells. By targeting CDKs and blocking their action, milciclib is expected to interfere with the cell division, eventually killing the cancer cells in malignant thymoma.

Milciclib also blocks a protein called TRKA that is thought to be overactive and stimulate growth of the cancer cells in malignant thymoma. This means that milciclib could have a dual action in controlling the disease.

What is the stage of development of this medicine?

The effects of milciclib maleate have been evaluated in experimental models.

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

At the time of submission, milciclib maleate was not authorised anywhere in the EU for the treatment of malignant thymoma. Orphan designation of milciclib maleate has been granted in the United States of America for treatment of thymic epithelial tumours.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 5 October 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:

Nerviano Medical Science Srl
Viale Pasteur 10
20014 Nerviano
Italy
Telephone: +39 03 31 58 11 11
Telefax: +39 0331 58 12 13
E-mail: orphan.drug@nervianoms.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[†], Norwegian and Icelandic

Language	Active substance	Indication
English	Milciclib maleate	Treatment of malignant thymoma
Bulgarian	Милциклиб малеат	Лечение на злокачествен тимом
Czech	Milciclib Maleate	Léčba maligních thymomů
Danish	Milciclibmaleat	Behandling af thymisk epitheliale tumorer
Dutch	Milciclibmaleaat	Behandling af malignt tymom
Estonian	Miltsikliibmaleaat	Tüümuse epiteelialsete kasvajate ravi
Finnish	Miltsiklibimaleaatti	Epiteelialisen tymooman hoito
French	Maléate de milciclib	Traitement des tumeurs épithéliales thymiques
German	Milciclib Maleate	Behandlung von malignen Thymomen
Greek	Μηλεϊνική μιλσικλίμπη	Θεραπεία του κακοήθους θυμώματος
Hungarian	Milciclib maleát	Malignus thymoma kezelése
Italian	Milciclib Maleato	Trattamento del timoma maligno
Latvian	Milcikliba maleāts	Ļaundabīgas timomas ārstēšana
Lithuanian	Milciklibo maleatas	Piktybinės timomos gydymas
Maltese	Milciclib maleate	Kura tat-tumuri epiteljali tat-timu
Polish	Maleinian milciklibu	Leczenie grasiczaka złośliwego
Portuguese	Maleato de Milciclib	Tratamento de tumores epiteliais do timo
Romanian	Milciclib maleat	Tratamentul tumorilor epiteliale timice maligne
Slovak	Maleát milciklibu	Léčba malígneho tymómu
Slovenian	Milciklib maleat	Zdravljenje malignega timoma
Spanish	Milciclib maleato	Tratamiento del timoma maligno
Swedish	Milciclib maleat	Behandling av epiteliala tumörer i tymus
Norwegian	Milciklibmaleat	Behandling av malignt tymom
Icelandic	Milkiklib maleat	Meðferð við æxlum í þekjufrumum hóstakirtils

[†] At the time of designation