10 November 2010
EMA/COMP/1290/03 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

ranpirnase for the treatment of malignant mesothelioma

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in August 2010 on request of the sponsor.

On 29 March 2001, orphan designation (EU/3/01/033) was granted by the European Commission to Dr. Erika Morgenstern, Germany, for ranpirnase for the treatment of malignant mesothelioma.

The sponsorship was transferred to MoRa Pharm GmbH, Germany in March 2003.

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 0.05 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 1,900 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).

[*]Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 377,000,000 (Eurostat 2001) and may differ from the true number of patients affected by the condition.

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What treatments are available?

No satisfactory methods exist that 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?

Ranpirnase is a preparation of a naturally occurring protein, extracted from eggs of the northern leopard frog. Experimental evidence indicates that ranpirnase binds to receptors on the surface of the cancer cells and enters the cell's interior. In the cell ranpirnase degrades RNA (a part of the fundamental genetic material of cells) and thereby inhibits production of proteins, cell growth and division, and may kill tumour cells.

What is the stage of development of this medicine?

The effects of ranpirnase were evaluated in experimental models. At the time of submission of the application for orphan designation, three clinical trials in patients with malignant mesothelioma were ongoing.

Ranpirnase was not marketed anywhere worldwide for 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 9 February 2001 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 languages[1], Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Ranpirnase	Treatment of malignant mesothelioma
Danish	Ranpirnase	Behandling af malign mesoteliom
Dutch	Ranpirnase	Behandeling van maligne mesotheliomen
Finnish	Ranpirnaasi	Pahanlaatuisen mesoteliooman hoito
French	Ranpirnase	Traitement du mésothéliome malin
German	Ranpirnase	Behandlung des malignen Mesothelioms
Greek	Ρανπιρνάση	Θεραπεία κακοήθους μεσοθηλιώματος
Italian	Ranpirnase	Trattamento del mesotelioma maligno
Portuguese	Ranpirnase	Tratamento do mesotelioma maligno
Spanish	Ranpirnase	Tratamiento del mesotelioma maligno
Swedish	Ranpirnase	Behandling av malignt mesoteliom

[1] At the time of designation