



Document Date: London, 29 November 2005
Doc.Ref.: EMEA/COMP/1640/02 Rev. 3

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in May 2005 on request from the sponsor.

COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF mitotane for the treatment of adrenal cortical carcinoma

On 11 September 2002, orphan designation (EU/3/02/113) was granted by the European Commission to Etablissement Pharmaceutique des Hôpitaux de Paris (AGEPS-EPHP), France, for mitotane for the treatment of adrenal cortical carcinoma.

What is adrenal cortical carcinoma?

Cancer of the adrenal cortex is a disease in which cancer (malignant) cells are found in the adrenal cortex, which is the outside layer of the adrenal gland. Cancer of the adrenal cortex is also called adrenal cortical carcinoma. There are two adrenal glands, one above each kidney in the back of the upper abdomen. The adrenal cortex produces steroid hormones. Steroid hormones include sex hormones and hormones used to control minerals and sugar in the body. Cancer cells in the adrenal cortex may make too much of one or more hormones, which can cause symptoms such as high blood pressure, weakening of the bones, or diabetes. If male or female hormones are affected, the body may go through changes such as a deepening of the voice, growing hair on the face, swelling of the sex organs, or swelling of the breasts. Cancers that make hormones are called functioning tumours. Many cancers of the adrenal cortex do not make extra hormones and are called non-functioning tumours. Adrenal cortical carcinoma is a life-threatening condition, mainly due to frequent spreading of the cancer cells (metastasis).

What are the methods of treatment available?

The methods of treatment used include surgery, chemotherapy (using drugs to kill the cancer cells) and radiotherapy (using x-rays or other high-energy rays to kill cancer cells). An anticancer medicinal product had been authorised for the treatment of the symptoms of the condition in the Community at the time of the submission of the application for orphan designation. However, there is no authorised treatment available to target adrenocortical carcinoma.

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

According to the information provided by the sponsor, adrenocortical carcinoma was considered to affect about 4,000 people in the European Union.

How is this medicinal product expected to act?

The potential of mitotane to be toxic to the adrenal cells, and therefore to limit tumour growth has been known since several decades but the exact mechanism of action is not known. Mitotane may be of potential interest for the treatment of adrenocortical carcinoma, particularly based on the effect, which have been shown in experimental models of the condition and in several small clinical studies.

What is the stage of development of this medicinal product?

At the time of submission of the application for orphan designation, clinical trials in patients with adrenal cortical carcinoma had been completed.

Mitotane had been marketed in the United States of America and Canada for the treatment of the condition at the time of the orphan application. Mitotane had not been designated as orphan medicinal product elsewhere for this condition, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 18 July 2002 a positive opinion recommending the grant of the above-mentioned designation.

Opinions on orphan medicinal products designations are based on the following cumulative criteria: (i) the seriousness of the condition, (ii) the existence or not of alternative methods of diagnosis, prevention or treatment and (iii) either the rarity of the condition (considered to affect not more than five in ten thousand persons in the Community) or the insufficient return of development investments.

Designated orphan medicinal products are still investigational products, which have been considered for designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of the quality, safety and efficacy will be necessary before this product can be granted a marketing authorisation.

For more information:

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*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. This estimate is based on available information and calculations presented by the sponsor at the time of the application.