The European Agency for the Evaluation of Medicinal Products
Pre-authorisation Evaluation of Medicines for Human Use

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF rubitecan for the treatment of pancreatic cancer

On 10 June 2003, orphan designation (EU/3/03/145) was granted by the European Commission to Eurogen Pharmaceuticals Limited, United Kingdom, for rubitecan for the treatment of pancreatic cancer.

What is pancreatic cancer?
Cancer that begins in the pancreas is called pancreatic cancer. The pancreas is a small organ that lies behind the stomach and in front of the spine. The pancreas has two main functions in the body. It makes a juice that helps to digest (break down) food. It produces hormones, such as insulin, that help to control blood sugar levels. About 95% of pancreatic cancers come from the cells that make the juice to digest. These cancers of the pancreas are called adenocarcinomas. Pancreatic cancer is life-threatening.

What are the methods of treatment available?
The choice of the treatment of pancreatic cancer depends on several factors, including the stage of the disease. Treatments may include surgery, radiation therapy (using high-dose x-rays or other high-energy rays to kill cancer cells), and chemotherapy (using drugs to kill cancer cells). There are anti-cancer drugs that have been authorised for treatment of pancreatic cancer. Rubitecan might be of potential significant benefit for the treatment of pancreatic cancer. It might offer a treatment to patients for whom other anti-cancer agents are not effective. This assumption remains to be proven. This will be necessary to maintain the orphan status.

What is the estimated number of patients affected by pancreatic cancer?
According to the information provided by the sponsor, pancreatic cancer was considered to affect between 30,000 and 40,000 persons in the European Union.

How is this medicinal product expected to act?
Rubitecan, belongs to a group of alkaloids called camptothecins. Alkaloids are substances that are naturally found in plants. Certain camptothecins are useful in medicine as anti-cancer agents. When cells are growing, as is the case for cancer cells, the genetic material (DNA) inside the cell may become twisted. Cells have several proteins which help to remove any twists in the DNA. This avoids that the DNA breaks, which would damage the cells. Camptothecins are able to block one of the proteins that can remove twists in the DNA. This protein is called topoisomerase I. By blocking this protein, rubitecan is expected to damage the cancer cells.

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 pancreatic cancer were ongoing.

Rubitecan was not marketed anywhere worldwide for pancreatic cancer or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

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According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 15 April 2003 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 were 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.

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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.