European Medicines Agency
*Pre-authorisation Evaluation of Medicines for Human Use*

**Document Date:** London, 22 February 2007
Doc.Ref.: EMEA/COMP/613/02 Rev. 2

***Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in November 2006 on request of the sponsor.***

## COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

# PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF bryostatin-1 for the treatment of oesophageal cancer

On 30 April 2002, orphan designation (EU/3/02/099) was granted by the European Commission to GPC Biotech AG, Germany, for bryostatin-1 for the treatment of oesophageal cancer.

**What is oesophageal cancer?**
Cancer that begins in the oesophagus (gullet) is called oesophageal cancer. The oesophagus is the muscular tube through which food passes from the throat to the stomach. Cancer of the oesophagus is often debilitating and life-threatening, despite available treatment.

**What are the methods of treatment available?**
Treatments for cancer of the oesophagus includes surgery as the most common treatment. Other treatments are chemotherapy and radiation therapy. Several products had been authorised for treatment of cancer of the oesophagus in the Community at the time of designation. Bryostatin-1 has a new mechanism of action. Bryostatin-1 used together with other drugs might be of potential significant benefit for the treatment of oesophageal cancer.

**What is the estimated number of patients affected by the condition*?**
According to the information provided by the sponsor, oesophageal cancer was considered to affect about 30,000 persons in the European Union.

**How is this medicinal product expected to act?**
Bryostatin-1 is derived from an alga that grows in the sea (*Bugula neritina*). This plant is also called sea moss, sea mat, or false coral. Bryostatin-1 interferes with a family of enzymes that are necessary for the cells to grow. These enzymes are called protein kinase C.

**What is the stage of development of this medicinal product?**
The effects of bryostatin-1 have been evaluated in experimental models. At the time of orphan designation, clinical trials in patients with cancer of the oesophagus were ongoing.

Bryostatin-1 had not been marketed anywhere worldwide at the time of designation. In the United States orphan drug status was granted for the treatment of this type of cancer.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 26 March 2002 a positive opinion recommending the grant of the above-mentioned designation.

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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.