European Medicines Agency
*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 masitinib mesilate for the treatment of pancreatic cancer

On 28 October 2009, orphan designation (EU//09/684) was granted by the European Commission to AB Science, France, for masitinib mesylate for the treatment of pancreatic cancer.

**What is pancreatic cancer?**
Pancreatic cancer is cancer of the pancreas, a small organ that lies behind the stomach. The pancreas has two functions: producing a juice that helps with the digestion of food, and producing hormones such as insulin. Due to the absence of symptoms in the early stages of the disease, the majority of patients are diagnosed when the cancer has spread locally or to other parts of the body.
Pancreatic cancer is a very severe and life-threatening disease that leads to poor long-term survival.

**What is the estimated number of patients affected by the condition?**
At the time of designation, pancreatic cancer affected approximately 1.3 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 66,000 people, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

**What treatments are available?**
At the time of submission of the application for orphan drug designation, several medicines were authorised for pancreatic cancer in the EU. The choice of treatment for pancreatic cancer depended on several factors, including how advanced the disease is. Treatments included surgery, radiotherapy (treatment with radiation) and chemotherapy (medicines to treat cancer).
The sponsor has provided sufficient information to show that masitinib mesylate might be of significant benefit for patients with pancreatic cancer because early studies indicate that it might improve the treatment of this condition when used in combination with existing treatments. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

**How is this medicine expected to work?**
Masitinib mesylate is expected to work by blocking types of enzymes known as tyrosine kinases. These enzymes can be found in some receptors on the surface of cancer cells, including 'c-Kit' receptors and 'platelet-derived growth factor' (PDGF) receptors. These are receptors involved in stimulating the cells to divide uncontrollably. By blocking these receptors, masitinib mesylate is expected to help to control cell division, slowing down the rate of growth of the 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. This represents a population of 504,800,000 (Eurostat 2009).

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**What is the stage of development of this medicine?**
The effects of masitinib mesylate have been evaluated in experimental models.
At the time of submission of the application for orphan designation, clinical trials in patients with pancreatic cancer were ongoing.
At the time of submission, masitinib mesylate was not authorised anywhere in the EU for pancreatic cancer or designated as orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 2 September 2009 recommending the granting of this designation.

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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 Community) 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.

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**Translations of the active ingredient and indication in all official EU languages, Norwegian and Icelandic**

| Language | Active ingredient | Indication |
|---|---|---|
| English | Masitinib mesilate | Treatment of pancreatic cancer |
| Bulgarian | Маситиниб месилат | Лечение на рак на панкреаса |
| Czech | Masitinibini mesylat | Léčba karcinomu pankreatu |
| Danish | Masitinibmesylat | Behandling af pancreascancer |
| Dutch | Masitinib mesilaat | Behandeling van pancreaskanker |
| Estonian | Masitiniibmesülaat | Pankreasevähi ravi |
| Finnish | Masitinibimesylaatti | Haimasyövän hoito |
| French | Masitinib mésylate | Traitement du cancer pancréatique |
| German | Masitinibmesilat | Behandlung des Pankreaskarzinoms |
| Greek | Masitinib μεσυλική | Θεραπεία καρκίνου του παγκρέατος |
| Hungarian | Maszitinib-mezilát | Hasnyálmirigyrák kezelése |
| Italian | Masitinib mesilato | Trattamento del cancro pancreatico |
| Latvian | Masitinība mezilāts | Aizkuņģa dziedzera vēža ārstēšana |
| Lithuanian | Mazitinibo mesilatas | Kasos vėžio gydymas |
| Maltese | Masitinib mesilate | Kura tal-kanċer tal-frixa |
| Polish | Mesylan masytynibu | Leczenie raka trzustki |
| Portuguese | Mesilato de masitinib | Tratamento do carcinoma do pâncreas |
| Romanian | Mesilat de masitinib | Tratamentul cancerului pancreatic |
| Slovak | Masitinib-mezylátu | Liečba rakoviny pankreasu |
| Slovenian | Masitinibov mesilat | Zdravljenje raka trebušne slinavke |
| Spanish | Mesilato de masitinib | Tratamiento del cáncer de páncreas |
| Swedish | Masitinibmesylat | Behandling av pankreascancer |
| Norwegian | Masitinibmesilat | Behandling av pankreascancer |
| Icelandic | Masitíníbmesýlat | Meðferð briskrabbameins |