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Questions and answers

Refusal of the marketing authorisation for Masiviera (masitinib)

Outcome of re-examination

On 23 January 2014, the Committee for Medicinal Products for Human Use (CHMP) adopted a negative opinion, recommending the refusal of the marketing authorisation for the medicinal product Masiviera, intended for the treatment of advanced inoperable pancreatic cancer. The company that applied for authorisation is AB Science.

The applicant requested a re-examination of the opinion. After considering the grounds for this request, the CHMP re-examined the initial opinion, and confirmed the refusal of the marketing authorisation on 22 May 2014.

What is Masiviera?

Masiviera is an anticancer medicine that contains the active substance masitinib. It was to be available as tablets.

What was Masiviera expected to be used for?

Masiviera was expected to be used to treat adults with cancer of the pancreas (an organ of the digestive system) that is locally advanced or metastatic (has spread to other parts of the body), non-resectable (unsuitable for surgery) and is accompanied by at least moderate pain. It was to be used in combination with another cancer medicine, gemcitabine.

Masitinib was designated an 'orphan medicine' (a medicine to be used in rare diseases) on 28 October 2009 for the treatment of pancreatic cancer. For more information, see here.

How is Masiviera expected to work?

The active substance in Masiviera, masitinib, is a tyrosine-kinase inhibitor. This means that it blocks certain enzymes known as tyrosine kinases. These enzymes can be found in some receptors on the surface of cells, including the receptors that are involved in stimulating cancer cells to divide

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uncontrollably. By blocking these receptors, Masiviera might help in controlling cell division and thereby slow down the growth of the cancer.

What did the company present to support its application?

The effects of Masiviera were first tested in experimental models before being studied in humans.

The company presented results of one main study involving 353 patients with advanced or metastatic pancreatic cancer. Masiviera was compared with placebo (a dummy treatment) as an addition to treatment with gemcitabine. The main measure of effectiveness was how long the patients survived with their disease. The company also presented various supporting analyses and information from a supportive study.

What were the CHMP's main concerns that led to the refusal?

At the time of the initial evaluation, the CHMP noted that the results of the main study with Masiviera did not show effectiveness in the overall group of patients with advanced or metastatic pancreatic cancer. Although the company presented analyses suggesting that there was a benefit in a subgroup of patients with certain genetic changes associated with more aggressive disease, and in a subgroup of patients with pain, the study was not designed to show benefit in these smaller groups, and the Committee considered that further study would be needed to demonstrate such a benefit. In addition, Masiviera was associated with significant toxicity. Furthermore, the CHMP had concerns about the quality of the product, in particular about the impurities to which patients might be exposed and about whether commercial batches of the medicine would have the same quality as those used for the studies.

During the re-examination the CHMP looked again at the data from the company, including a proposal for a conditional authorisation in a restricted group of patients, and also consulted a group of experts in the treatment of pancreatic cancer. The Committee confirmed its opinion that the effectiveness of Masiviera had not been sufficiently demonstrated in pancreatic cancer. In addition, some concerns about the quality of the medicine were not yet resolved. Therefore, the CHMP concluded that the benefits of Masiviera did not outweigh its risks and maintained the previous recommendation that the medicine be refused marketing authorisation.

What consequences does this refusal have for patients in clinical trials or compassionate use programmes?

The company informed the CHMP that there were no consequences for patients in clinical trials or compassionate use programmes.

If you are in a clinical trial or compassionate use programme and need more information about your treatment, contact the doctor who is giving it to you.