EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Questions and answers

Refusal of the marketing authorisation for Masican (masitinib)

Outcome of re-examination

On 21 November 2013, the Committee for Medicinal Products for Human Use (CHMP) adopted a negative opinion, recommending the refusal of the marketing authorisation for the medicinal product Masican, intended for the treatment of malignant gastrointestinal stromal tumour (GIST). 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 20 March 2014.

What is Masican?

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

What was Masican expected to be used for?

Masican was expected to be used to treat gastrointestinal stromal tumour (GIST), a cancer of the stomach and bowel, in adults whose cancer cannot be removed surgically or has spread and is getting worse despite treatment with imatinib, another medicine used to treat this cancer.

Masican was designated an 'orphan medicine' (a medicine to be used in rare diseases) on 21 December 2004 for the treatment of malignant GIST. For more information, see here.

How is Masican expected to work?

The active substance in Masican, 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

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surface of cancer cells, including the receptors that are involved in stimulating the cells to divide uncontrollably. By blocking these receptors, Masican 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 applicant presented results of a main study in 44 patients with GIST that could not be removed surgically or that had spread and was resistant to imatinib treatment. The study included a group of patients treated with Masican and one group treated with sunitinib, another medicine of the same class. The study was a 'phase II study', an exploratory study designed to see if a medicine is worth investigating further. The main measure of effectiveness was progression-free survival (how long the patients lived without the disease getting worse).

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

At the time of the initial evaluation, the CHMP was concerned that the study was not designed to compare Masican with sunitinib and that it was difficult to interpret the results. Although the group of patients treated with Masican appeared to live longer than those treated with sunitinib, the possibility that this was a chance finding could not be ruled out because of the exploratory nature of the study and because other supportive evidence was lacking. Therefore, the Committee concluded that there were insufficient data to establish the benefits of Masican.

The CHMP was also concerned that safety data were only available for a few patients treated with Masican at the proposed dose and this did not allow a proper evaluation of the medicine's side effects. Finally, there were concerns about the quality control of the medicine during manufacture, which led to uncertainties about the impurities that patients would be exposed to.

During the re-examination, although the medicine's safety was of less concern, the Committee still had major concerns about the medicine's benefits. In addition, there were still outstanding problems related to quality control during its manufacture. Therefore the CHMP concluded that the benefits of Masican 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?

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.