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**Questions and answers**

# Refusal of the marketing authorisation for Masipro (masitinib)

On 18 May 2017, the Committee for Medicinal Products for Human Use (CHMP) adopted a negative opinion, recommending the refusal of the marketing authorisation for the medicinal product Masipro, intended for the treatment of systemic mastocytosis.

The company that applied for authorisation is AB Science. It may request a re-examination of the opinion within 15 days of receipt of notification of this negative opinion.

## What is Masipro?

Masipro is a medicine that contains the active substance masitinib. It was to be available as tablets.

## What was Masipro expected to be used for?

Masipro was expected to be used to treat adults with systemic mastocytosis, a disease in which there are too many mast cells (a type of white blood cell) in the skin, bones and various body organs, causing symptoms such as itchy skin, hot flushes, palpitations, fainting, bone pain, weakness, vomiting, diarrhoea and depression.

Masipro was designated an 'orphan medicine' (a medicine to be used in rare diseases) on 16 November 2004 for mastocytosis. Further information on the orphan designation can be found here.

## How does Masipro work?

The active substance in Masipro, masitinib, is a tyrosine kinase inhibitor. This means that it blocks enzymes known as tyrosine kinases, which can be found in some receptors in mast cells including those involved in stimulating the cells to grow and divide. By blocking these enzymes, Masipro helps to slow down the growth of the mast cells.

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## What did the company present to support its application?

The applicant presented data from a main study involving 135 patients with systemic mastocytosis who had severe symptoms including at least one of the following: itching, hot flushes, depression and weakness. In the study, Masipro was compared with placebo (a dummy treatment). The main measure of effectiveness was based on improvements in any of the four symptoms mentioned above after 24 weeks of treatment.

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

The CHMP was concerned about the reliability of the study results because a routine GCP (good clinical practice) inspection at the study sites revealed serious failings in the way the study had been conducted. In addition, major changes were made to the study design while the study was ongoing, which made the results difficult to interpret. Finally, data on the safety of the medicine were limited and there were concerns regarding the medicine's side effects, including neutropenia (low levels of white blood cells) and harmful effects on the skin and liver, which were of relevance particularly because the medicine was to be used long term.

Therefore, at that point in time, the CHMP was of the opinion that the benefits of Masipro did not outweigh its risks and recommended that it 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 are 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.