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Refusal of the marketing authorisation for Masitinib AB Science (masitinib mesilate)

Re-examination confirms refusal

After re-examining its initial opinion, the European Medicines Agency has confirmed its recommendation to refuse marketing authorisation for the medicine Masitinib AB Science, a medicine intended for the treatment of amyotrophic lateral sclerosis (ALS).

The Agency issued its opinion after re-examination on 17 October 2024. The Agency had issued its initial opinion on 27 June 2024. The company that applied for authorisation of Masitinib AB Science is AB Science.

What is Masitinib AB Science and what was it intended for?

Masitinib AB Science was developed as a medicine to treat adults with ALS. It was intended to be used in combination with riluzole (another medicine for ALS). ALS is a progressive disease of the nervous system, where nerve cells in the brain and spinal cord that control voluntary movement gradually deteriorate, causing loss of muscle function and paralysis.

Masitinib AB Science contains the active substance masitinib mesilate and was to be available as tablets.

Masitinib AB Science was designated an 'orphan medicine' (a medicine used in rare diseases) on 29 August 2016 for the treatment of ALS. Further information on the orphan designation can be found on the Agency's website: ema.europa.eu/medicines/human/orphan-designations/eu3161722.

How does Masitinib AB Science work?

The active substance in Masitinib AB Science, masitinib mesilate, is a protein-kinase inhibitor. This means that it blocks specific enzymes known as protein kinases. Blocking these enzymes affects the activity of certain cells of the immune system (the body's natural defences) involved in inflammation. By blocking these enzymes, masitinib mesilate was expected to reduce inflammation and protect nerve cells from damage, thereby slowing worsening of ALS symptoms.

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What did the company present to support its application?

The company presented results from a main study involving 394 adults with ALS. Patients were given Masitinib AB Science or placebo (a dummy treatment) twice a day for 48 weeks. All patients also received riluzole. The main measure of effectiveness was the change over 48 weeks in the ALSFRS-R score, a measure of ALS symptoms that affect patients' daily living.

What were the main reasons for refusing the marketing authorisation?

At the time of the initial evaluation, the Agency considered that the study data were not reliable, as findings from good clinical practice (GCP) inspections coordinated by EMA and other regulatory authorities had identified issues with the conduct of the study that could not be sufficiently addressed by the company. In addition, the benefits of Masitinib AB Science could not be convincingly demonstrated; the study found no difference between the medicine and placebo in the main measure of effectiveness for the total study population and had several methodological issues.

These concerns did not change after re-examination of the data provided and following consultation of a group of experts in neurology, as well as patient representatives. In reaching its final decision, the Agency also considered information shared by patient organisations (so-called third-party interventions).

Therefore, the Agency maintained its opinion that the benefits of Masitinib AB Science did not outweigh its risks and recommended refusing marketing authorisation.

Does this refusal affect patients in clinical trials?

The company informed the Agency that there are no consequences for patients in clinical trials with Masitinib AB Science.

If you are in a clinical trial and need more information about your treatment, speak with your clinical trial doctor.