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Questions and answers

Withdrawal of the application for a change to the marketing authorisation for Macugen (pegaptanib)

On 15 July 2011, Pfizer officially notified the Committee for Medicinal Products for Human Use (CHMP) that it wishes to withdraw its application for a new indication, the treatment of visual impairment due to diabetic macular oedema.

What is Macugen?

Macugen is a solution for injection into the eye that contains the active substance pegaptanib. It is available as a prefilled syringe.

Macugen has been authorised since January 2006. It is already used to treat patients with the 'wet' form of age-related macular degeneration (AMD). This disease affects the central part of the retina (called the macula) at the back of the eye and causes loss of 'straight-ahead' vision.

What was Macugen expected to be used for?

Macugen was also expected to be used to treat visual impairment in patients with diabetic macular oedema (DME). This is the swelling of the macular caused by diabetes.

How is Macugen expected to work?

In diabetic macular oedema, Macugen is expected to work in the same way as it does in its existing indication. The active substance in Macugen, pegaptanib, is an 'aptamer'. An aptamer is a single strand of molecules called nucleotides that has been designed to attach to a structure in the body. Pegaptanib has been designed to attach to a substance called vascular endothelial growth factor (VEGF) and to block its action. In the body, VEGF is involved in the growth of blood vessels and in making them more permeable. Pegaptanib injected into the eye blocks VEGF. This reduces the growth of blood vessels and controls the leakage and swelling.

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What did the company present to support its application?

The applicant presented an overview of the effects of Macugen in experimental models from previous studies as well as some additional data.

The company also presented results from a main study in 317 patients with diabetic macular oedema. Patients were either treated with Macugen or given a 'sham' injection, in which a syringe is pressed against the eye with nothing actually injected. The main measure of effectiveness was the number of patients after one year who could read 10 more letters than they previously could in a standard eye test. The patients were tested again at the end of the second year.

How far into the evaluation was the application when it was withdrawn?

The application was withdrawn after 'day 90'. This means that the CHMP had evaluated the initial documentation provided by the company and formulated lists of questions. The CHMP was assessing the company's responses to the questions at the time of the withdrawal.

What was the recommendation of the CHMP at that time?

Based on the review of the data and the company's response to the CHMP list of questions, at the time of the withdrawal, the CHMP had concerns mainly about the effect of the treatment. The CHMP's main concern was that the effect of Macugen in the main study was not convincing, in particular the results at the end of the second year where Macugen was not proven to be more effective than the sham injection. The improvements seen at the end of the first year were not maintained. Therefore, at the time of the withdrawal, the CHMP was not fully convinced that the benefits of Macugen outweigh its risks in patients with visual impairment due to diabetic macular oedema.

What were the reasons given by the company for withdrawing the application?

The letter from the company notifying the Agency of the withdrawal of the application is available under the tab 'All documents'.

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

The company informed the CHMP that, as at the time of the withdrawal, there were no consequences for patients currently included in clinical trials using Macugen. If you are in a clinical trial and need more information about your treatment, contact the doctor who is giving it to you.

What is happening with Macugen for the treatment of patients with the 'wet' form of age-related macular degeneration (AMD)?

There are no consequences on the use of Macugen in its authorised indication.

The full European Public Assessment Report for Macugen searched for on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European Public Assessment Reports.