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Questions and answers

Withdrawal of the marketing authorisation application for Opsiria (sirolimus)

On 20 May 2016, Santen Oy officially notified the Committee for Medicinal Products for Human Use (CHMP) that it wishes to withdraw its application for a marketing authorisation for Opsiria, for the treatment of non-infectious uveitis.

What is Opsiria?

Opsiria is a medicine that contains the active substance sirolimus. It was to be available as a solution to be injected into the eye.

What was Opsiria expected to be used for?

Opsiria was expected to be used for the treatment of non-infectious uveitis (inflammation of the uvea, the middle layer of the eye) in adults. The inflammation may cause discomfort, pain, blurring of vision, and may lead to partial or complete blindness.

Opsiria was designated an 'orphan medicine' (a medicine to be used in rare diseases) on 30 August 2011 for the treatment of chronic non-infectious uveitis. Further information can be found here: ema.europa.eu/Find medicine/Human medicines/Rare disease designation.

How is Opsiria expected to work?

The active substance in Opsiria, sirolimus, is expected to work by blocking an enzyme called 'mammalian target of rapamycin' (mTOR). Since mTOR is involved in the activation and proliferation of T lymphocytes (white blood cells that play a role in inflammation), sirolimus is expected to reduce the inflammation in chronic non-infectious uveitis.

Sirolimus has been used for several years to prevent organ rejection following a transplant.

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What did the company present to support its application?

Opsiria was investigated in one main study in 347 patients with non-infectious uveitis. In this study, Opsiria was not compared with any other treatment. The main measure of effectiveness was the number of patients in whom inflammation resolved after 5 months of treatment.

How far into the evaluation was the application when it was withdrawn?

The application was withdrawn after that the CHMP had evaluated the documentation provided by the company and formulated lists of questions. After the CHMP had assessed the company's responses to the last round of questions, there were still some unresolved issues.

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 lists of questions, at the time of the withdrawal, the CHMP had had some concerns and was of the provisional opinion that Opsiria could not have been approved for the treatment of non-infectious uveitis. The data from the clinical study was not sufficient to demonstrate the benefit of Opsiria, particularly in European patients. In addition, the Committee questioned the proposed method of sterilisation of the medicine.

Therefore, at the time of the withdrawal, the CHMP was of the opinion that the benefits of Opsiria did not outweigh its risks.

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

In its letter notifying the Agency of the withdrawal of application, the company acknowledged the need to submit additional data on the benefits of Opsiria from an ongoing clinical study.

The withdrawal letter is available here.

What consequences does this withdrawal have for patients in clinical trials?

The company informed the CHMP that there are no consequences for patients currently included in clinical trials using Opsiria.

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