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Withdrawal of application to change the marketing authorisation for Ilaris (canakinumab)

Novartis withdrew its application for the use of Ilaris in the treatment of Schnitzler syndrome.

The company withdrew the application on 26 October 2022.

What is Ilaris and what is it used for?

Ilaris is a medicine used to treat several inflammatory conditions, including Still's disease, gouty arthritis and several types of periodic fever syndromes. It has been authorised in the EU since October 2009.

It contains the active substance canakinumab and is given as an injection under the skin. Further information on Ilaris' current uses can be found on the Agency's website: https://www.ema.europa.eu/en/medicines/human/EPAR/ilaris

What change had the company applied for?

The company applied to extend the use of Ilaris to treat Schnitzler syndrome, a rare long-term inflammatory disease causing urticaria (hives), recurrent fever, bone and joint pain, and swollen lymph nodes.

How does Ilaris work?

In patients with Schnitzler syndrome, Ilaris is expected to work in the same way as it does in its existing indications. The active substance in Ilaris, canakinumab, is a monoclonal antibody, a type of protein that has been designed to recognise and attach to a messenger molecule or 'cytokine' in the body called interleukin-1 beta. This messenger is involved in causing inflammation and is found in high levels in patients with inflammatory conditions. By attaching to interleukin-1 beta, canakinumab blocks its activity, helping to reduce inflammation thereby relieving the symptoms of the disease.

What did the company present to support its application?

The company presented the results of a main study in 20 patients with Schnitzler syndrome comparing the effects of Ilaris with those of placebo. The study looked at the proportion of patients who had no or minimal disease activity after 7 days of treatment, based on the assessment of 5 key symptoms. In

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addition the company presented supportive evidence from additional smaller studies described in the literature.

How far into the evaluation was the application when it was withdrawn?

The application was withdrawn after the European Medicines Agency had evaluated the information from the company and had prepared questions for the company. After the Agency had assessed the company's responses to the questions, there were still some unresolved issues.

What did the Agency recommend at that time?

Based on the review of the information and the company's response to the Agency's questions, at the time of the withdrawal, the Agency had some concerns and its provisional opinion was that Ilaris could not have been authorised for the use in Schnitzler syndrome.

The Agency's main concerns were about the way the main study had been conducted, which raised questions about the accuracy and validity of the data. In particular, the Agency considered the quality of documentation insufficient to carry out an adequate assessment and found errors in the randomisation process affecting the accuracy and reliability of results.

Therefore, at the time of the withdrawal, the Agency's opinion was that it was not possible to come to reliable conclusions on how well the medicine works in the treatment of Schnitzler syndrome and the Agency therefore considered that the benefits of Ilaris does not outweigh its risks for this use.

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 stated that the withdrawal is based on the fact that the available data were not considered sufficient to conclude on a benefit-risk balance for the proposed indication.

Does this withdrawal affect patients in clinical trials?

The company informed the Agency that there are no consequences for patients in clinical trials using Ilaris.

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

What is happening with Ilaris for the treatment of other diseases?

There are no consequences on the use of Ilaris in its authorised uses.