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Withdrawal of application to change the marketing authorisation for Ongentys (opicapone)

BIAL withdrew its application for the use of Ongentys in the treatment of signs and symptoms of Parkinson's disease.

The company withdrew the application on 12 March 2024.

What is Ongentys and what is it used for?

Ongentys is a medicine used for treating adults with Parkinson's disease, a progressive brain disorder that causes shaking and muscle stiffness, and slows movement.

Ongentys is used as an add-on to levodopa / DOPA decarboxylase inhibitors (DDCI) (other medicines for Parkinson's disease) in patients who are having motor fluctuations in the control of their condition. Fluctuations happen when the effects of the medication wear off and symptoms re-emerge before the next dose is due. Ongentys is used when these fluctuations cannot be treated with the standard levodopa-containing combinations alone.

Ongentys has been authorised in the EU since June 2016.

It contains the active substance opicapone and is available as capsules to be taken by mouth.

Further information on Ongentys's current uses can be found on the Agency's website: ema.europa.eu/en/medicines/human/EPAR/ongentys

What change had the company applied for?

The company applied to extend the use of Ongentys to treat signs and symptoms of Parkinson's disease; this means that its use in addition to levodopa / DDCI was to be extended to a broader group of patients, including patients with early stage Parkinson's disease. It was no longer to be restricted to patients having (motor) fluctuations in the control of their condition.

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How does Ongentys work?

In patients with Parkinson's disease, the cells in the brain that produce the neurotransmitter dopamine begin to die and the amount of dopamine in the brain decreases. The patients then lose their ability to control their movements reliably. The active substance in Ongentys, opicapone, works to restore the levels of dopamine in the parts of the brain that control movement and coordination. It enhances the effects of levodopa, a copy of the neurotransmitter dopamine that can be taken by mouth. Opicapone blocks an enzyme that is involved in the breakdown of levodopa in the body called catechol-O-methyl transferase (COMT). As a result, levodopa remains active for longer. This helps to improve the symptoms of Parkinson's disease, such as stiffness and slowness of movement.

What did the company present to support its application?

The company presented data from a study involving 355 patients with early stage Parkinson's disease who received either Ongentys or placebo (a dummy treatment) in addition to levodopa / DDCI. Patients in the study had no signs of any motor complications (meaning fluctuations in their response to treatment or involuntary movements). The main measure of effectiveness was an improvement in motor symptoms as measured using a standard scale called Movement Disorder Society-Unified Parkinson's Disease Rating Scale (MDS-UPDRS).

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 Ongentys could not have been authorised for the treatment of signs and symptoms of early stage Parkinson's disease.

The Agency noted that the data on the effectiveness of Ongentys were not robust enough and indicated only a small effect of the medicine on MDS-UPDRS score and other measures of effectiveness. It was therefore not clear whether patients with mild symptoms of early stage Parkinson's disease would benefit from treatment with Ongentys.

Therefore, at the time of the withdrawal, the Agency's opinion was that, because effectiveness was not proven in the broader use, the benefits of Ongentys 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 stated that they withdrew their application due to the need to conduct additional work to fully address the questions raised by the Agency.

Does this withdrawal affect patients in clinical trials?

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

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

What is happening with Ongentys in its authorised use?

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