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Withdrawal of application for the marketing authorisation of Viokat (diazoxide choline)

Soleno Therapeutics Europe Limited withdrew its application for a marketing authorisation of Viokat for the treatment of hyperphagia (extreme hunger that cannot be satisfied) in people with Prader-Willi syndrome.

The company withdrew the application on 6 April 2026.

What is Viokat and what was it intended to be used for?

Viokat was developed as a medicine to treat hyperphagia in adults and children from 4 years of age with Prader-Willi syndrome. Prader-Willi syndrome is a genetic condition that affects growth, development and behaviour. People with the condition also develop a constant feeling of hunger, which can lead to serious health problems if not well managed.

Viokat contains the active substance diazoxide choline and was to be available as prolonged-release tablets to be taken by mouth once daily.

Viokat was designated an 'orphan medicine' (a medicine used in rare diseases) on 8 November 2017 for the treatment of Prader-Willi syndrome. Further information on the orphan designation can be found on the Agency's website: ema.europa.eu/medicines/human/orphan-designations/eu-3-17-1941.

How does Viokat work?

The active substance of Viokat, diazoxide choline, works by activating potassium channels in nerve cells in a certain area of the brain, the hypothalamus, that produces two types of proteins, the neuropeptide Y and the agouti-related protein, involved in stimulating appetite. Activation of potassium channels in the nerve cells reduces the level of these proteins, which is expected to reduce the feeling of hunger in people with Prader-Willi syndrome.

What did the company present to support its application?

The company presented results from two main studies, with the second study involving patients who had completed the first one.

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The first main study involved 127 adults and children from 4 years of age with Prader-Willi syndrome who had hyperphagia. Patients in the study received either Viokat or placebo (a dummy treatment) once daily. The main measure of effectiveness was the change in the total score on the hyperphagia questionnaire for clinical trials (HQ-CT) after 13 weeks of treatment.

The second main study carried out in two phases. The first phase involved 115 patients who had completed the first main study and who took Viokat for up to 5 years. The second phase involved 77 patients who had completed the first phase and who either continued to take Viokat or who stopped treatment and received a placebo for 16 weeks. The main measure of effectiveness was the change in HQ-CT score after these 16 weeks.

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 prepared questions for the company. The company had responded to the last round of questions but withdrew the application before the Agency assessed the responses.

What did the Agency recommend at that time?

Based on the review of the data, at the time of the withdrawal, the Agency had concerns and its provisional opinion was that Viokat could not have been authorised for the treatment of hyperphagia in people with Prader-Willi syndrome.

The Agency considered that the first study failed to meet its main endpoint. While the second study, involving patients from the first study, met its main endpoint, the Agency considered that the population was highly selected. As there were also concerns about the design of the study, these results were insufficient to demonstrate the effectiveness of the medicine.

Concerning quality, the Agency considered that, based on the information provided, it was not possible to rule out the formation of nitrosamine impurities (impurities that could potentially cause cancer).

Therefore, at the time of the withdrawal, the Agency's opinion was that the company had not fully addressed its concerns and the benefit of Viokat could not be established.

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

In its letter notifying the Agency of the withdrawal of the application, the company stated that the decision to withdraw followed the preliminary assessment by the CHMP, which concluded that, the data provided were not sufficient to determine that the benefits of the medicine outweigh its risks.

Does this withdrawal affect patients in clinical trials or compassionate use programmes?

The company informed the Agency that there are no consequences for patients in clinical trials or in compassionate use programmes using Viokat.

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