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Withdrawal of application for the marketing authorisation of Pelgraz Paediatric (pegfilgrastim)

Accord Healthcare S.L.U. withdrew its application for a marketing authorisation of Pelgraz Paediatric to treat neutropenia (low levels of neutrophils, a type of white blood cell that helps to fight infections) and prevent febrile neutropenia (neutropenia accompanied by fever) in children with cancer. Neutropenia is a common side effect of cancer chemotherapy and increases the risk of infections.

The company withdrew the application on 20 February 2025. This withdrawal does not affect the marketing authorisation for Pelgraz which is authorised to treat neutropenia in adults with cancer.

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

Pelgraz Paediatric was developed as a medicine to reduce the duration of neutropenia and prevent febrile neutropenia in children with cancer who weigh between 10 kg and 45 kg.

Pelgraz Paediatric contains the active substance pegfilgrastim and was to be available as a pre-filled syringe containing a solution for injection under the skin, given as a single dose after each chemotherapy cycle.

Pelgraz Paediatric was developed as a 'biosimilar' medicine. This means that Pelgraz Paediatric was intended to be highly similar to another biological medicine containing pegfilgrastim already authorised in the EU (the 'reference medicine'). The reference medicine for Pelgraz Paediatric is Neulasta, which is authorised for use only in adults whereas Pelgraz Paediatric was developed specifically for use in children (a paediatric use marketing authorisation). For more information on biosimilar medicines, see here.

How does Pelgraz Paediatric work?

The active substance in Pelgraz Paediatric and Neulasta, pegfilgrastim, is a form of filgrastim, which is very similar to a human protein called granulocyte colony stimulating factor (G-CSF). Like G-CSF, filgrastim works by encouraging the bone marrow to produce more white blood cells, increasing white blood cell counts, so preventing and treating neutropenia, and helping the body fight infections.

Filgrastim has been available in other medicines in the European Union for a number of years. In Pelgraz Paediatric and Neulasta, filgrastim has been 'pegylated' (attached to a chemical called

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polyethylene glycol). This slows down the removal of filgrastim from the body, allowing the medicine to be given less often.

What did the company present to support its application?

The company presented studies to show that Pelgraz Paediatric is comparable to the reference medicine in adults. Because Pelgraz Paediatric was expected to be used in children, for which the reference medicine was not authorised, the company presented results from a main study involving 12 children under 6 years of age receiving chemotherapy for cancer. The study compared Pelgraz Paediatric with a non-pegylated formulation of filgrastim.

The company also provided other data including data from the scientific literature and two meta-analyses (combined analyses of several published studies) to support the effectiveness and safety of pegfilgrastim in children at the proposed dosing schedule.

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. After the Agency had assessed the company's responses to the last round of questions, there were still some unresolved issues.

What did the Agency recommend at that time?

Based on the review of the data 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 Pelgraz Paediatric could not have been authorised for the reduction of neutropenia and prevention of febrile neutropenia in children with cancer and weighing between 10 kg and 45 kg.

The Agency had concerns about the proposed dosing schedule not being sufficiently supported by data. In addition, there were concerns about the accuracy of the dosing with the pre-filled syringe, which could lead to dosing errors. Therefore, at the time of the withdrawal, the Agency's opinion was that the benefits of Pelgraz Paediatric 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 the application, the company stated that they were withdrawing the application due to the concerns 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 Pelgraz Paediatric.

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